

# HUMAN GENE TRANSFER PROTOCOLS

Last updated: 9-13-04

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**8810-001 (Closed) Gene Marking/Cancer/In Vitro/Tumor Infiltrating Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous-**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *The Treatment of Patients with Advanced Cancer Using Cyclophosphamide, Interleukin-2 and Tumor Infiltrating Lymphocytes.*

\*RAC Recommends Approval: 10-3-88/NIH Approval: 3-2-89  
Closed: 4-04-96

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**9007-002 (Closed) Gene Therapy/Phase I/Monogenic Disease/Severe Combined Immune Deficiency due to Adenosine Deaminase Deficiency/In Vitro/Autologous Peripheral Blood Cells/CD34+ Autologous Peripheral Blood Cells/Cord Blood/Placenta Cells/Retrovirus/Adenosine Deaminase cDNA/Neomycin Phosphotransferase cDNA/Intravenous**

Blaese, R. Michael; National Institutes of Health, Bethesda, Maryland; *Treatment of Severe Combined Immune Deficiency (SCID) due to Adenosine Deaminase (ADA) Deficiency with Autologous Lymphocytes Transduced with the Human ADA Gene: An Experimental Study.*

\*RAC Recommends Approval: 7-31-90/NIH Approval: 9-6-90

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**9007-003 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/n Vitro/Tumor Infiltrating Lymphocytes/Retrovirus/Cytokine/Tumor Necrosis Factor cDNA/Neomycin Phosphotransferase cDNA/Intravenous**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Gene Therapy of Patients with Advanced Cancer Using Tumor Infiltrating Lymphocytes Transduced with the Gene Coding for Tumor Necrosis Factor.*

\*RAC Recommends Approval: 7-31-90/NIH Approval: 9-6-90  
Closed: 6-26-96

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**9102-004 (Closed) Gene Marking/Cancer/Acute Myelogenous Leukemia/n Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Brenner, Malcolm K.; Mirro, Joseph; Hurwitz, Craig; Santana, Victor; and Ihle, James; St. Jude Children's Research Hospital, Memphis, Tennessee; *Autologous Bone Marrow Transplant for Children with Acute Myelogenous Leukemia in First Complete Remission: Use of Marker Genes to Investigate the Biology of Marrow Reconstitution and the Mechanism of Relapse.*

\*RAC Recommends Approval: 2-4-91/NIH Approval: 7-12-91  
Closed: 1-21-93

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**9105-005 (Closed) Gene Marking/Cancer/Neuroblastoma/n Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Brenner, Malcolm K.; Mirro, Joseph; Santana, Victor; and Ihle, James; St. Jude Children's Research Hospital, Memphis, Tennessee; *A Phase I/II Trial of High Dose Carboplatin and Etoposide with Autologous Marrow Support for Treatment of Stage D Neuroblastoma in First Remission: Use of Marker Genes to Investigate the Biology of Marrow Reconstitution and the Mechanism of Relapse.*

\*RAC Recommends Approval: 5-31-91/NIH Approval: 7-12-91  
Closed: 9-1-92

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**9105-006 (Closed) Gene Marking/Cancer/Neuroblastoma/n Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Brenner, Malcolm K.; Mirro, Joseph; Santana, Victor; and Ihle, James; St. Jude Children's Research Hospital, Memphis, Tennessee; *A Phase II Trial of High-Dose Carboplatin and Etoposide with Autologous Marrow Support for Treatment of Relapse/Refractory Neuroblastoma Without Apparent Bone Marrow Involvement.*

\*RAC Recommends Approval: 5-31-91/NIH Approval: 7-12-91  
Closed: 4-9-93

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\*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

**9105-007 (Closed) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Deisseroth, Albert B.; M.D. Anderson Cancer Research Center, Houston, Texas; *Autologous Bone Marrow Transplantation for Chronic Myelogenous Leukemia in which Retroviral Markers are Used to Discriminate between Relapse which Arises from Systemic Disease Remaining after Preparative Therapy Versus Relapse due to Residual Leukemic Cells in Autologous Marrow: A Pilot Trial.*

\*RAC Recommends Approval: 5-31-91/NIH Approval: 7-12-91

Closed: 6-1-93

Closed: 4-9-93

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**9105-008 (Closed) Gene Marking/Acute Hepatic Failure/In Vitro/Autologous Hepatocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intrahepatic**

Ledley, Fred D.; Woo, Savio; Ferry, George; and Hartwell, Whisennand; Baylor College of Medicine, Houston, Texas; *Hepatocellular Transplantation in Acute Hepatic Failure and Targeting Genetic Markers to Hepatic Cells.*

\*RAC Recommends Approval: 5-30-91/NIH Approval: 7-12-91

Closed: Protocol Never Initiated

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**9105-009 (Closed) Gene Marking/Cancer/Melanoma/In Vitro/Tumor Infiltrating Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous**

Lotze, Michael T.; University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *The Administration of Interleukin-2 and Tumor Infiltrating Lymphocytes to Patients with Melanoma.*

\*RAC Recommends Approval: 5-30-91/NIH Approval: 1-17-92

Closed: 4-95

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**9110-010 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Renal Cell/Colon/Breast/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Tumor Necrosis Factor cDNA/Neomycin Phosphotransferase cDNA/Subcutaneous Injection**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Immunization of Cancer Patients Using Autologous Cancer Cells Modified by Insertion of the Gene for Tumor Necrosis Factor (TNF).*

\*RAC Recommends Approval: 10-7-91/NIH Approval: 10-15-91

Closed: 4-15-96

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**9110-011 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Renal Cell/Colon/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Immunization of Cancer Patients Using Autologous Cancer Cells Modified by Insertion of the Gene for Interleukin-2 (IL-2).*

\*RAC Recommends Approval: 10-7-91/NIH Approval: 10-15-91

Closed: 4-15-96

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**9110-012 (Closed) Gene Therapy/Phase I/Monogenic Disease/Familial Hypercholesterolemia/In Vitro/Low Density Lipoprotein Receptor cDNA/Intrahepatic/Portal Vein Catheter**

Wilson, James M.; University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *Ex Vivo Gene Therapy of Familial Hypercholesterolemia.*

\*RAC Recommends Approval: 10-8-91/NIH Approval: 11-14-91

Closed: 3-11-94

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**9202-013 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Adenocarcinoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DC-Chol/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral/Direct Injection/Catheter Delivery to Pulmonary Nodules**

Nabel, Gary J.; University of Michigan, Ann Arbor, Michigan; *Immunotherapy of Malignancy by In Vivo Gene Transfer into Tumors.*

\*RAC Recommends Approval: 2-10-92/NIH Approval: 4-17-92

Closed: 11-19-92 (Replaced by Protocol #9306-045)

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**9202-014 (Closed) Gene Marking/Cancer/Acute Myelogenous Leukemia/Acute Lymphocytic Leukemia/In Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Cornetta, Kenneth; Indiana University, Indianapolis, Indiana; *Retroviral-Mediated Gene Transfer of Bone Marrow Cells during Autologous Bone Marrow Transplantation for Acute Leukemia.*

\*RAC Recommends Approval: 2-11-92/NIH Approval: 4-17-92  
Closed 5-1-95

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**9202-015 (Closed) Gene Marking/Cancer/Melanoma/Renal Cell/In Vitro/CD4+ Autologous Peripheral Blood Lymphocytes/CD8+ Autologous Peripheral Blood Lymphocytes/CD4+ Autologous Tumor Infiltrating Lymphocytes/CD8+ Autologous Tumor Infiltrating Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous**

Economou, James S. and Beldegrun, Arie; University of California at Los Angeles, Los Angeles, California; *The Treatment of Patients with Metastatic Melanoma and Renal Cell Cancer Using In Vitro Expanded and Genetically-Engineered (Neomycin Phosphotransferase) Bulk, CD8 (+) and/or CD4(+) Tumor Infiltrating Lymphocytes and Bulk, CD8(+) and/or CD4(+) Peripheral Blood Leukocytes in Combination with Recombinant Interleukin-2 Alone, or with Recombinant Interleukin-2 and Recombinant Alpha Interferon.*

\*RAC Recommends Approval: 2-11-92/NIH Approval: 4-17-92  
Closed: 6-94

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**9202-016 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intraperitoneal Administration**

Freeman, Scott M.; Tulane University Medical Center, New Orleans, Louisiana; *Gene Transfer for the Treatment of Cancer.*

\*RAC Recommends Approval: 2-10-92/NIH Approval: 2-5-93

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**9202-017 (Open) Gene Therapy/Infectious Disease/Human Immunodeficiency Virus/In Vitro/CD8+ Allogeneic Cytotoxic T Lymphocytes/CD8+ Syngeneic Cytotoxic T Lymphocytes/Retrovirus/Hygromycin Phosphotransferase/Herpes Simplex Virus Thymidine Kinase cDNA/Intravenous**

Greenberg, Philip D. and Riddell, Stanley; Fred Hutchinson Cancer Research Center, University of Washington, Seattle; *Phase I Study to Evaluate the Safety of Cellular Adoptive Immunotherapy Using Genetically Modified CD8+ HIV-Specific T Cells in HIV Seropositive Individuals.*

\*RAC Recommends Approval: 2-11-92/NIH Approval: 4-17-92

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**9206-018 (Closed) Gene Therapy/Phase I/Cancer/Relapsed-Refractory Neuroblastoma/Immunotherapy/In Vitro/Autologous Neuroblastoma Cells/Allogeneic Partially HLA-Matched/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection**

Brenner, Malcolm K.; Furman, Wayne; Santana, Victor; Bowman, Laura; and Meyer, William; St. Jude Children's Research Hospital, Memphis, Tennessee; *Phase I Study of Cytokine-Gene Modified Autologous Neuroblastoma Cells for Treatment of Relapsed/Refractory Neuroblastoma.*

\*RAC Recommends Approval: 6-1-92/NIH Approval: 8-14-92  
Closed to accrual: 1-29-02

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**9206-019 (Closed) Gene Therapy/Phase I/Cancer/Brain/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Stereotactic Injection**

Oldfield, Edward; National Institutes of Health, Bethesda, Maryland; *Gene Therapy for the Treatment of Brain Tumors Using Intra-Tumoral Transduction with the Thymidine Kinase Gene and Intravenous Ganciclovir.* Sponsor: Genetic Therapy, Inc./Novartis

\*RAC Recommends Approval: 6-1-92/NIH Approval: 8-14-92  
Closed: 12-94

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**9206-020 (Closed) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/Autologous Bone Marrow Cells/Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Deisseroth, Albert B.; M.D. Anderson Cancer Center, Houston, Texas; *Use of Two Retroviral Markers to Test Relative Contribution of Marrow and Peripheral Blood Autologous Cells to Recovery After Preparative Therapy.*

\*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92  
Closed: 2-13-96

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**9206-021 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Partially HLA-Matched/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection**

Gansbacher, Bernd; Houghton, Alan; and Livingston, Philip; Memorial Sloan Kettering Cancer Center, New York, New York; *Immunization with HLA-A2 matched Allogeneic Melanoma Cells that Secrete Interleukin-2 in Patients with Metastatic Melanoma.*

\*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92  
Closed: 10-19-94

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**9206-022 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vitro/Allogeneic Partially HLA-Matched/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection**

Gansbacher, Bernd; Motzer, Robert; Houghton, Alan; and Bander, Neil; Memorial Sloan Kettering Cancer Center, New York, New York; *Immunization with Interleukin-2 Secreting Allogeneic HLA-A2 Matched Renal Cell Carcinoma Cells in Patients with Advanced Renal Cell Carcinoma.*

\*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92

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**9206-023 (Closed) Gene Marking/Cancer/Multiple Myeloma/In Vitro/CD34+ Autologous Peripheral Blood Cells/Intravenous/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Dunbar, Cynthia; National Institutes of Health, Bethesda, Maryland; *Retroviral-Mediated Gene Transfer of Bone Marrow and Peripheral Blood Stem Cells During Autologous Bone Marrow Transplantation for Multiple Myeloma.*

\*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92  
Closed to accrual: December 1996

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**9206-024 (Closed) Gene Marking/Cancer/Breast/In Vitro/CD34+ Autologous Peripheral Blood Cells/Intravenous/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Dunbar, Cynthia; National Institutes of Health, Bethesda, Maryland; *Retroviral-Mediated Gene Transfer of Bone Marrow and Peripheral Blood Stem Cells During Autologous Bone Marrow Transplantation for Metastatic Breast Cancer.*

\*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92  
Closed to accrual: October 1996

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**9206-025 (Closed) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/Intravenous/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Dunbar, Cynthia; National Institutes of Health, Bethesda, Maryland; *Retroviral-Mediated Gene Transfer of Bone Marrow and Peripheral Blood Stem Cells During Autologous Bone Marrow Transplantation for Chronic Myelogenous Leukemia.*

\*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92

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**9209-026 (Closed) Gene Marking/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Syngeneic Peripheral Blood Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous**

Tavel, Jorge; National Institutes of Health, Bethesda, Maryland; *A Study of the Safety and Survival of the Adoptive Transfer of Genetically Marked Syngeneic Lymphocytes in HIV Infected Identical Twins.*

\*RAC Recommends Approval: 9-14-92/NIH Approval: 9-3-93  
Closed to new enrollment. Individuals will be followed, long-term, in a new protocol (that does not involve administration of recombinant DNA): 1-17-02

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**9209-027 (Closed) Gene Marking/Cancer/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Schuening, Friedrich G.; Miller, A. Dusty; and Kiem, Hans-Peter; Fred Hutchinson Cancer Research Center, University of Washington, Seattle, Washington; *Study on Contribution of Genetically Marked Peripheral Blood Repopulating Cells to Hematopoietic Reconstitution after Transplantation.*

\*RAC Recommends Approval: 9-14-92/NIH Approval: 2-5-93  
Closed: 4-29-97

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**9209-028 (Closed) Gene Marking/Cancer/Lymphoid Malignancies/In Vitro/G-CSF Mobilized Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Schuening, Friedrich G.; Fred Hutchinson Cancer Research Center, University of Washington, Seattle, Washington; *Evaluation of the Use of Recombinant Human G-CSF Stimulated Peripheral Blood Progenitor Cell Supplementation in Autologous Bone Marrow Transplantation in Patients with Lymphoid Malignancies.*

\*RAC Recommends Approval: 9-14-92/NIH Approval: 2-5-93  
Closed: 2-25-94 (Merged with protocol # 9209-027)

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**9209-029 (Closed) Gene Marking/Cancer/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Schuening, Friedrich G.; Fred Hutchinson Cancer Research Center, University of Washington, Seattle, Washington; *A Trial of G-CSF Stimulated Peripheral Blood Stem Cells for Engraftment in Identical Twins.*

\*RAC Recommends Approval: 9-14-92/NIH Approval: 2-5-93  
Closed: Protocol Never Initiated

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**9209-030 (Open) Gene Marking/Cancer/Chronic Lymphocytic Leukemia/Follicular Non-Hodgkin's Lymphoma/In Vitro/Autologous Bone Marrow Cells/Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Deisseroth, Albert B.; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Use of Retroviral Markers to Identify Efficacy of Purging and Origin of Relapse Following Autologous Bone Marrow and Peripheral Blood Cell Transplantation in Indolent B Cell Neoplasms (Follicular Non-Hodgkin's Lymphoma or Chronic Lymphocytic Leukemia) Patients.*

\*RAC Recommends Approval: 9-14-92/NIH Approval: 12-2-93

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**9403-031 (Open) Gene Therapy/Phase I/Cancer/Non-small Cell Lung Cancer/Antisense/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Retrovirus/p53 cDNA/kras Antisense/Intratumoral/Bronchoscope**

Roth, Jack A.; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; and Garver, Robert I., Jr.; University of Alabama at Birmingham, Birmingham, AL; *Clinical Protocol for Modification of Oncogene and Tumor Suppressor Gene Expression in Non-Small Cell Lung Cancer (NSCLC).*

\*RAC Recommends Approval: 3-4-94/NIH Approval: 1-4-95

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**9209-032 (Closed) Gene Marking/Cancer/Neuroblastoma/In Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Brenner, Malcolm K.; St. Jude Children's Research Hospital, Memphis, Tennessee; *A Phase II Trial of the Baxter Neuroblastoma Bone Marrow Purging System Using Gene Marking to Assess Efficacy.*

\*RAC Recommends Approval: 9-15-92/NIH Approval: 2-5-93

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**9209-033 (Open) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/In Combination with Untransduced Autologous Tumor Cells/Retrovirus/Cytokine/Interleukin-4 cDNA/Subcutaneous Injection**

Lotze, Michael T. and Rubin, Joshua T.; University of Pittsburgh, Pittsburgh, Pennsylvania; *Gene Therapy of Cancer: A Pilot Study of IL-4 Gene Modified Antitumor Vaccines.*

\*RAC Recommends Approval: 9-15-92/NIH Approval: 2-5-93

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**9212-034 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)**

Crystal, Ronald G.; Rockefeller University Hospital, New York, New York; *A Phase I Study, in Cystic Fibrosis Patients, of the Safety, Toxicity, and Biological Efficacy of a Single Administration of a Replication Deficient, Recombinant Adenovirus Carrying the cDNA of the Normal Human Cystic Fibrosis Transmembrane Conductance Regulator Gene in the Lung.*

\*RAC Recommends Approval: 12-3-92/NIH Approval: 4-16-93  
Protocol closed, IND inactive: 5-30-00

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**9212-035 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adenovirus/Serotype 5/E2a Temperature Sensitive Mutant/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)**

Wilson, James M., University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; Simon, Richard H., University of Michigan Medical Center, Ann Arbor, Michigan; McCoy, Karen, Cystic Fibrosis Center at Ohio State University; *Gene Therapy of Cystic Fibrosis Lung Diseases Using E1 Deleted Adenoviruses: A Phase I Trial.*

\*RAC Recommends Approval: 12-3-92/NIH Approval: 8-26-93  
No additional enrollment planned: 8-05-99

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**9212-036 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Adenovirus/Serotype 2/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal**

Welsh, Michael J.; Howard Hughes Medical Institute, Iowa City, Iowa; and Smith, Alan E.; Genzyme Corporation, Framingham, Massachusetts; *Cystic Fibrosis Gene Therapy Using an Adenovirus Vector: In Vivo Safety and Efficacy in Nasal Epithelium.* Sponsor: Genzyme Corporation

\*RAC Recommends Approval: 12-4-92/NIH Approval: 4-16-93  
Protocol ended in November 1993

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**9303-037 (Closed) Gene Therapy/Phase I/Cancer/Glioblastoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratatumoral/Direct Injection**

Van Gilder, John C.; University of Iowa, Iowa City, Iowa; Berger, Mitchell; University of California, San Francisco, California; Prados, Michael; University of Washington, Seattle, Washington; Warnick, Ronald; University of Cincinnati Medical Center, Cincinnati, Ohio; Schold, Clifford; University of Texas Southwestern Medical Center, Dallas, Texas; Fetell, Michael; Columbia Presbyterian Medical Center, New York, New York; Schramm, Johannes; Neurochirurgische Universitätsklinik, Bonn, Germany; Westphal, Manfred; University Clinic Eppendorf, Hamburg, Germany; Tonn, Jorg-Christian; University Kliniken, Würzburg, Germany; Mouldjian, Robert; Notre-Dame Hospital, Montreal, Quebec, Canada; Shaffrey, Mark; University of Virginia, Charlottesville, Virginia; Asher, Anthony; Charlotte Neurological Associates and Presbyterian Hospital, Charlotte, North Carolina; Epstein, Mel; Brown University, Providence, Rhode Island; Schmitz-Schackert, Gabriele Anna Maria; University Klinikum Karl-Gustav-Carus, Dresden, Germany; Mendez, Ivar; Victoria General Hospital, Nova Scotia, Canada; Bernstein, Mark; The Toronto Hospital, Toronto, Ontario, Canada; *Gene Therapy for the Treatment of Recurrent Glioblastoma Multiforme with In Vivo Tumor Transduction with the Herpes Simplex Thymidine Kinase Gene/Ganciclovir System.* Sponsor: Genetic Therapy, Inc./Novartis

\*RAC Recommends Approval: 3-1-93/NIH Approval: 4-16-93

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**9303-038 (Closed) Gene Marking/Cancer/Leukemia/Non-malignant Disorders/In Vitro/Epstein-Barr Virus Specific Allogeneic Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Heslop, Helen E.; Brenner, Malcolm K.; and Rooney, Cliona; St. Jude Children's Research Hospital, Memphis, Tennessee; *Administration of Neomycin Resistance Gene Marked EBV Specific Cytotoxic T Lymphocytes to Recipients of Mismatched-Related or Phenotypically Similar Unrelated Donor Marrow Grafts.*

\*RAC Recommends Approval: 3-2-93/NIH Approval: 4-16-93  
Gene Marking portion discontinued: February 1996

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**9303-039 (Closed) Gene Marking/Cancer/Acute Myelogenous Leukemia/In Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Brenner, Malcolm K.; Krance, Robert; Heslop, Helen E.; Santana, Victor; and Ihle, James; St. Jude Children's Research Hospital, Memphis, Tennessee; *Assessment of the Efficacy of Purging by Using Gene-Marked Autologous Marrow Transplantation for Children with Acute Myelogenous Leukemia in First Complete Remission.*

\*RAC Recommends Approval: 3-2-93/NIH Approval: 4-16-93

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**9303-040 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Subcutaneous Injection**

Simons, Jonathan; Johns Hopkins Oncology Center, Baltimore, Maryland; *Phase I Study of Non-Replicating Autologous Tumor Cell Injections Using Cells Prepared With or Without Granulocyte-Macrophage Colony Stimulating Factor Gene Transduction in Patients with Metastatic Renal Cell Carcinoma.*

\*RAC Recommends Approval: 3-1-93/NIH Approval: 12-2-93  
Study closed, long-term follow-up continues: 7-16-01

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**9303-041 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)**

Wilmott, Robert W. and Whitsett, Jeffrey; Children's Hospital Medical Center, Cincinnati, Ohio; and Trapnell, Bruce; Genetic Therapy, Inc., Gaithersburg, Maryland; *A Phase I Study of Gene Therapy of Cystic Fibrosis Utilizing a Replication Deficient Recombinant Adenovirus Vector to Deliver the Human Cystic Fibrosis Transmembrane Conductance Regulator cDNA to the Airways*. Sponsor: Genetic Therapy, Inc./Novartis

\*RAC Recommends Approval: 3-2-93/NIH Approval: 4-16-93  
Closed: 4-28-97 (IND Withdrawn)

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**9303-042 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator CDNA/Intranasal**

Boucher, Richard C. and Knowles, Michael R.; University of North Carolina, Chapel Hill, North Carolina; *Gene Therapy for Cystic Fibrosis Using E1 Deleted Adenovirus: A Phase I Trial in the Nasal Cavity*.

\*RAC Recommends Approval: 3-2-93/NIH Approval: 10-7-93  
Closed: 10-94

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**9306-043 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Gamma Interferon cDNA/Subcutaneous Injection**

Seigler, Hilliard F.; Duke University Medical Center, Durham, North Carolina; and Merritt, James A.; Viagene, Inc., San Diego, California; *A Phase I Trial of Human Gamma Interferon-Transduced Autologous Tumor Cells in Patients With Disseminated Malignant Melanoma*.

\*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93  
Closed to further enrollment: September 1997

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**9306-044 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Chemoprotection/In Vitro/CD34+ Autologous Bone Marrow Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Bone Marrow Transplant**

Deisseroth, Albert B.; Kavanagh, John; and Champlin, Richard; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Use of Safety-Modified Retroviruses to Introduce Chemotherapy Resistance Sequences into Normal Hematopoietic Cells for Chemoprotection During the Therapy of Ovarian Cancer: A Pilot Trial*.

\*RAC Recommends Approval: 6-7-93/NIH Approval: 12-2-93

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**9306-045 (Closed) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral/Direct Injection/Catheter Delivery to Pulmonary Nodules**

Nabel, Gary J.; University of Michigan Medical Center, Ann Arbor, Michigan; *Immunotherapy for Cancer by Direct Gene Transfer into Tumors*.

\*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93

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**9306-046 (Closed) Gene Therapy/Phase I/Monogenic Disease/Gaucher Disease/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Glucocerebrosidase cDNA/Bone Marrow Transplant**

Barranger, John A.; University of Pittsburgh, Pittsburgh, Pennsylvania; *Gene Therapy for Gaucher Disease: Ex Vivo Gene Transfer and Autologous Transplantation of CD34(+) Cells*.

\*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93

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**9306-047 (Closed) Gene Therapy/Phase I/Monogenic Disease/Gaucher Disease/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Glucocerebrosidase cDNA/Bone Marrow Transplant**

Karlsson, Stefan and Dunbar, Cynthia; National Institutes of Health, Bethesda, Maryland; and Kohn, Donald B.; Childrens Hospital Los Angeles, Los Angeles, California; *Retroviral Mediated Transfer of the cDNA for Human Glucocerebrosidase into Hematopoietic Stem Cells of Patients with Gaucher Disease*. Sponsor: Genetic Therapy, Inc./Novartis

\*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93  
Closed: 4-30-97 (IND Withdrawn)

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**9306-048 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1IIIB Envelope Protein/Intramuscular Injection**

Galpin, Jeffrey E.; University of Southern California; Casciato, Dennis A.; Shared Medical Research Foundation, Tarzana, California; and Merritt, James A.; Viagene, Inc., San Diego, California; *A Preliminary Study to Evaluate the Safety and Biologic Effects of Murine Retroviral Vector Encoding HIV-1 Genes [HIV-IT(V)] in Asymptomatic Subjects Infected with HIV-1*. Sponsor: Chiron Corporation

\*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93  
Closed: 9-8-94

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**9306-049 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Retrovirus/Particle Mediated Gene Transfer (Accell®)/RSV-tar/Rev M10/Intravenous**

Nabel, Gary J.; University of Michigan Medical Center, Ann Arbor, Michigan; *A Molecular Genetic Intervention for AIDS - Effects of a Transdominant Negative Form of Rev*.

\*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93

IND terminated: 3-13-00

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**9306-050 (Open) Gene Therapy/Phase I/Cancer/Astrocytoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Ommaya Injection**

Raffel, Corey; Mayo Clinic, Rochester, Minnesota; Villablanca, Judith; Childrens Hospital Los Angeles, Los Angeles, California; Packer, Roger, Childrens National Medical Center, Washington, DC; Tonn, Jorg-Christian, Neurochirurgische Klinik und Poliklinik, Universitäts-Klinik, Würzburg, Germany; and Burdach, Stefan; University Center for Paediatrics, Heinrich-Heine Universität, Düsseldorf, Germany; *Gene Therapy for the Treatment of Recurrent Pediatric Malignant Astrocytomas with In Vivo Tumor Transduction with the Herpes Simplex Thymidine Kinase Gene*. Sponsor: Genetic Therapy, Inc./Novartis

\*RAC Recommends Approval: 6-8-93/NIH Approval: 9-3-93

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**9306-051 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Brain/Chemoprotection/In Vitro/CD34+ Autologous Bone Marrow Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Bone Marrow Transplant**

Hesdorffer, Charles and Antman, Karen; Columbia University College of Physicians and Surgeons, New York, New York; *Human MDR Gene Transfer in Patients with Advanced Cancer*.

\*RAC Recommends Approval: 6-8-93/NIH Approval: 9-3-93

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**9306-052 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma/Antisense/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Lipofectin (Gibco BRL)/Insulin-like Growth Factor Antisense/Subcutaneous Injection**

Ilan, Joseph; Case Western Reserve University School of Medicine and University Hospitals of Cleveland, Cleveland, Ohio; *Gene Therapy for Human Brain Tumors Using Episome-Based Antisense cDNA Transcription of Insulin-Like Growth Factor I*.

\*RAC Recommends Approval: 6-8-93/NIH Approval: 12-2-93

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**9309-053 (Open) Gene Therapy/Phase I/Cancer/Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Lipofectin (Gibco BRL)/Cytokine/Interleukin-2 cDNA/Neomycin Phosphotransferase cDNA/Subcutaneous Injection**

Podack, Eckhard R.; Sridhar, Kasi; University of Miami; and Savaraj, Niramol; Miami Veterans Administration Hospital, Miami, Florida; *Phase I Study of Transfected Cancer Cells Expressing the Interleukin-2 Gene Product in Limited Stage Small Cell Lung Cancer*.

\*RAC Recommends Approval: 9-9-93/NIH Approval: 12-2-93

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**9309-054 (Closed) Gene Therapy/Phase I/Cancer/Breast/Chemoprotection/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Intravenous**

O'Shaughnessy, Joyce; Kentuckiana Medical Oncology Association, Louisville, Kentucky; *Retroviral Mediated Transfer of the Human Multi-Drug Resistance Gene (MDR-1) into Hematopoietic Stem Cells During Autologous Transplantation after Intensive Chemotherapy for Breast Cancer*.

\*RAC Recommends Approval: 9-9-93/NIH Approval: 10-7-93  
Study completed, follow-up continues: 5-31-00

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**9309-055 (Closed) Gene Therapy/Phase I/Cancer/Brain Tumors/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection**

Kun, Larry E.; Sanford, R. A.; Brenner, Malcolm K.; and Heideman, Richard L.; St. Jude Childrens Research Hospital, Memphis, Tennessee; and Oldfield, Edward H.; National Institutes of Health, Bethesda, Maryland; *Gene Therapy for Recurrent Pediatric Brain Tumors*. Sponsor: Genetic Therapy, Inc./Novartis

\*RAC Recommends Approval: 9-9-93/NIH Approval: 10-7-93  
Closed to accrual: July 1997

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**9309-056 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Interleukin-2 cDNA/Neomycin Phosphotransferase cDNA/Subcutaneous Injection**

Das Gupta, Tapas K. and Cohen, Edward P.; University of Illinois at Chicago, Chicago, Illinois; *Immunization of Malignant Melanoma Patients with Interleukin 2-Secreting Melanoma Cells Expressing Defined Allogeneic Histocompatibility Antigens*.

\*RAC Recommends Approval: 9-10-93/NIH Approval: 4-19-94

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**9309-057 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus-1/Replication Inhibition/Hairpin Ribozyme/In Vitro/CD4+ Peripheral Blood Cells/Retrovirus/Hairpin Ribozyme/Intravenous**

Wong-Staal, Flossie; Poeschla, Eric; and Looney, David; University of California, San Diego, California; *A Phase I Clinical Trial to Evaluate the Safety and Effects in HIV-1 Infected Humans of Autologous Lymphocytes Transduced with a Ribozyme that Cleaves HIV-1 RNA*.

\*RAC Recommends Approval: 9-10-93/NIH Approval: 10-25-94

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**9309-058 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/In Combination with Untransduced Autologous Tumor Cells/Retrovirus/Interleukin-2 cDNA/Subcutaneous Injection**

Economou, James S. and Glaspy, John A.; University of California Medical Center, Los Angeles, California; *Genetically Engineered Autologous Tumor Vaccines Producing Interleukin-2 for the Treatment of Metastatic Melanoma*.

\*RAC Recommends Approval: 9-10-93/NIH Approval: 12-2-93

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**9312-059 (Closed) Gene Therapy/Phase I/Cancer/Leptomeningeal Carcinomatosis/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intraventricular Injection/Subarachnoid Injection**

Oldfield, Edward H. and Ram, Zvi; National Institutes of Health, Bethesda, Maryland; *Intrathecal Gene Therapy for the Treatment of Leptomeningeal Carcinomatosis*. Sponsor: Genetic Therapy, Inc./Novartis

\*RAC Recommends Approval: 12-2-93/NIH Approval: 1-20-94  
Closed: 1/95

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**9312-060 (Open) Gene Therapy/Phase I/Cancer/Colon/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/In Combination with Untransduced Autologous Tumor Cells/Retrovirus/Interleukin-2 cDNA/Subcutaneous Injection**

Sobol, Robert E. and Royston, Ivor; San Diego Regional Cancer Center, San Diego, California; *Injection of Colon Carcinoma Patients with Autologous Irradiated Tumor Cells and Fibroblasts Genetically Modified to Secrete Interleukin-2*.

\*RAC Recommends Approval: 12-2-93/NIH Approval: 1-4-95

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**9312-061 (Closed) Gene Therapy/Phase I/Monogenic Disease/Gaucher Disease/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/Glucocerebrosidase cDNA/Intravenous**

Schuening, Friedrich; Fred Hutchinson Cancer Research Center, Seattle, Washington; *Retrovirus-Mediated Transfer of the cDNA for Human Glucocerebrosidase into Peripheral Blood Repopulating Cells of Patients with Gaucher's Disease*.

\*RAC Recommends Approval: 12-2-93/NIH Approval: 11-15-94  
Closed: 4-29-97

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**9312-062 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1IIIB Envelope Protein/Intramuscular Injection**

Haubrich, Richard; University of California at San Diego Treatment Center, San Diego, California; and Merritt, James A.; Viagene, Inc., San Diego, California; *An Open Label, Phase I/II Clinical Trial to Evaluate the Safety and Biological Activity of HIV-IT(V) (HIV-1 IIIIBenv/rev Retroviral Vector) in HIV-1 Infected Subjects.*

\*RAC Recommends Approval: 12-3-93/NIH Approval: 4-19-94  
Closed: 10-13-94

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**9312-063 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Lipofectin (Gibco BRL)/B7 (CD80) cDNA/Neomycin Phosphotransferase cDNA/Subcutaneous Injection**

Sznol, Mario; National Institutes of Health, Frederick, Maryland; *A Phase I Trial of B7-Transfected Lethally Irradiated Allogeneic Melanoma Cell Lines to Induce Cell Mediated Immunity Against Tumor-Associated Antigens Presented by HLA-A2 or HLA-A1 in Patients with Stage IV Melanoma.*

\*RAC Recommends Approval: 12-3-93/NIH Approval: 4-19-94

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**9312-064 (Closed) Gene Therapy/Phase I/Cancer/Colon/Hepatic Metastases/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral/Hepatic Injection**

Rubin, Joseph; Mayo Clinic, Rochester, Minnesota; *Phase I Study of Immunotherapy of Advanced Colorectal Carcinoma by Direct Gene Transfer into Hepatic Metastases.* Sponsor: Vical, Incorporated

\*RAC Recommends Approval: 12-3-93/NIH Approval: 4-19-94  
Closed: 3-16-95 (Closed to accrual - maximum number of subjects entered)

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**9312-065 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Used in Combination with Anti-CD3 and Interleukin-2 Primed Autologous Lymph Node Cells to Prime Autologous Peripheral Blood Cells In Vitro/Retrovirus/GM-CSF cDNA/Intravenous**

Chang, Alfred E.; University of Michigan, Ann Arbor, Michigan; *Adoptive Immunotherapy of Cancer with Activated Lymph Node Cells Primed In Vivo with Autologous Tumor Cells Transduced with the GM-CSF Gene.*

\*RAC Recommends Approval: 12-3-93/NIH Approval: 8-23-94

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**9312-066 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cationic Liposome Complex/DMRIE-DOPE/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal**

Sorscher, Eric J. and Logan, James L.; University of Alabama, Birmingham, Alabama; *Gene Therapy for Cystic Fibrosis Using Cationic Liposome Mediated Gene Transfer: A Phase I Trial of Safety and Efficacy in the Nasal Airway.*

\*RAC Recommends Approval: 12-3-93/NIH Approval: 1-4-95  
Closed: May 2000

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**9312-067 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Maxillary Sinus Epithelial Cells/Adenovirus/Serotype 2/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Maxillary Sinus Administration**

Welsh, Michael J.; Howard Hughes Medical Institute, Iowa City, Iowa; *Adenovirus-Mediated Gene Transfer of CFTR to the Nasal Epithelium and Maxillary Sinus of Patients with Cystic Fibrosis.* Sponsor: Genzyme Corporation

\*RAC Recommends Approval: 12-3-93/NIH Approval: 2-10-94  
Protocol ended in May 1995

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**9403-068 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Gamma Interferon cDNA/Subcutaneous Injection**

Rosenblatt, Joseph; University of California, Los Angeles, California; Seeger, Robert; Childrens Hospital, Los Angeles, California; and Merritt, James A.; Viagene, Inc., San Diego, California; *A Phase I Study of Immunization with Gamma Interferon Transduced Neuroblastoma Cells.*

\*RAC Recommends Approval: 3-3-94/NIH Approval: 10-25-94

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**9403-069 (Closed) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vitro/CD8+ Syngeneic Peripheral Blood Cells/Retrovirus/CD4-zeta Chimeric Receptor/Intravenous/Concurrent Interleukin-2 Therapy**

Walker, Robert; National Institutes of Health, Bethesda, Maryland; *A Phase I/II Pilot Study of the Safety of the Adoptive Transfer of Syngeneic Gene-Modified Cytotoxic T-Lymphocytes in HIV-Infected Identical Twins*. Sponsor: NIH/Cell Genesys, Inc.

\*RAC Recommends Approval: 3-3-94/NIH Approval: 8-23-94  
Closed: 2-97

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**9403-070 (Open) Gene Therapy/Phase I/Monogenic Disease/Alpha-1-Antitrypsin Deficiency/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Cationic Liposome Complex/DC-Chol-DOPE/Alpha-1 Antitrypsin cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)**

Brigham, Kenneth; Clinical Research Center at Vanderbilt University Medical Center, Nashville, Tennessee; *Expression of an Exogenously Administered Human Alpha-1-Antitrypsin Gene in the Respiratory Tract of Humans*. Sponsor: Gene Medicine, Inc.

\*RAC Recommends Approval: 3-3-94/NIH Approval: 10-25-94

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**9403-071 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral/Direct Injection**

Vogelzang, Nicholas; the University of Chicago, Chicago, Illinois; *Phase I Study of Immunotherapy for Metastatic Renal Cell Carcinoma by Direct Gene Transfer into Metastatic Lesions*. Sponsor: Vical, Incorporated

\*RAC Recommends Approval: 3-4-94/NIH Approval: 4-19-94  
Closed: 4-5-95 (Closed to accrual - maximum number of subjects entered)

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**9403-072 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin/cDNA/Intratumoral/Direct Injection**

Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; and Akporiaye; Harris; Stopeck; Unger; and Warneke; University of Arizona, Tucson, Arizona; *Phase I Study of Immunotherapy of Malignant Melanoma by Direct Gene Transfer*. Sponsor: Vical, Incorporated

\*RAC Recommends Approval: 3-4-94/NIH Approval: 4-19-94  
Closed: 3-27-95 (Closed to accrual - maximum number of subjects entered)

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**9406-073 (Open) Gene Therapy/Phase I/Colon/Immunotherapy/In Vivo/Autologous Tumor Cells/Plasmid DNA/Carcinoembryonic Antigen Plasmid Expression Vector/Kanamycin Resistance cDNA/Intratumoral/Direct Injection**

Curiel, David; University of Alabama, Birmingham, Alabama; *Phase I Trial of a Polynucleotide Augmented Anti-Tumor Immunization to Human Carcinoembryonic Antigen in Patients with Metastatic Colorectal Cancer*.

\*RAC Recommends Approval: 6-10-95/NIH Approval: 7-27-95

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**9406-074 (Closed) Gene Therapy/Phase I/Other/Rheumatoid Arthritis/In Vivo/Autologous Synovial Cells/Retrovirus/Interleukin-1 Receptor Antagonist Protein cDNA/Intrajoint/Metacarpal Phalangeal Joints**

Evans, C. H. and Robbins, Paul; University of Pittsburgh, Pittsburgh, Pennsylvania; *Clinical Trial to Assess the Safety, Feasibility, and Efficacy of Transferring a Potentially Anti-arthritis Cytokine Gene to Human Joints with Rheumatoid Arthritis*.

\*RAC Recommends Approval: 6-9-94/NIH Approval: 7-27-95

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**9406-075 (Closed) Gene Marking/Cancer/Ovarian/In Vitro/Autologous Peripheral Blood Cells/Autologous Tumor Infiltrating Lymphocytes/Retrovirus/Neomycin Phosphotransferase/cDNA/Intraperitoneal**

Freedman, Ralph; M.D. Anderson Cancer Center, Houston, Texas; *Use of a Retroviral Vector to Study the Trafficking Patterns of Purified Ovarian TIL Populations Used in Intraperitoneal Adoptive Immunotherapy of Ovarian Cancer Patients: A Pilot Study*.

\*RAC Recommends Approval: 6-9-94/NIH Approval: 7-12-94  
No subjects entered, protocol withdrawn

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**9406-076 (Closed) Gene Marking/Cancer/Pediatric Malignancies/In Vitro/CD34+ Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Heslop, Helen; Brenner, Malcolm, K.; and Krance, Robert; St. Jude Childrens Research Hospital, Memphis, Tennessee; *Use of Double Marking with Retroviral Vectors to Determine the Rate of Reconstitution of Untreated and Cytokine Expanded CD34(+) Selected Marrow Cells in Patients Undergoing Autologous Bone Marrow Transplantation.*

\*RAC Recommends Approval: 6-9-94/NIH Approval: 7-12-94  
Closed: November 1997

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**9406-077 (Closed) Gene Therapy/Phase II/Cancer/Breast/Chemoprotection/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Intravenous**

Deisseroth, Albert; Hortobagyi, Gabriel; Champlin, Richard; and Holmes, Frankie; M.D. Anderson Cancer Center, Houston, Texas; *Use of Safety-Modified Retroviruses to Introduce Chemotherapy Resistance Sequences into Normal Hematopoietic Cells for Chemoprotection During the Therapy of Breast Cancer: A Pilot Trial.*

\*RAC Recommends Approval: 6-9-94/NIH Approval: 7-12-94

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**9406-078 (Closed) Gene Therapy/Phase II/Monogenic Disease/Fanconi Anemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Fanconi Anemia Complementation Group C cDNA/Intravenous**

Liu, Johnson, M. and Young, Neal S.; National Institutes of Health, Bethesda, Maryland; and Wagner, John E., University of Minnesota, Minneapolis, Minnesota; *Retroviral Mediated Gene Transfer of the Fanconi Anemia Complementation Group C Gene to Hematopoietic Progenitors of Group C Patients.*

\*RAC Recommends Approval: 6-9-94/NIH Approval: 2-12-95  
Closed: 1997, follow-up continuing

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**9406-079 (Closed) Gene Therapy/Phase II/Cancer/Non-small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral/Bronchoscope**

Roth, Jack A.; M.D. Anderson Cancer Center, Houston, Texas; *Clinical Protocol for Modification of Tumor Suppressor Gene Expression and Induction of Apoptosis in Non-Small Cell Lung Cancer (NSCLC) with an Adenovirus Vector Expressing Wildtype p53 and Cisplatin.*

\*RAC Recommends Approval: 6-10-94 and 9-11-95/NIH Approval: 9-21-95  
Closed: December 1997

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**9406-080 (Open) Gene Therapy/Phase II/Cancer/Glioblastoma/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/In Combination with Untransduced Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection**

Sobol, Robert and Royston, Ivor; San Diego Regional Cancer Center; San Diego, California; *Injection of Glioblastoma Patients with Tumor Cells Genetically Modified to Secrete Interleukin-2 (IL-2): A Phase I Study.*

\*RAC Recommends Approval: 6-10-94/NIH Approval: 7-12-94

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**9406-081 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Lymphoma/Breast/Head and Neck Cancer/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-12 cDNA/Neomycin Phosphotransferase cDNA/Intratumoral/Direct Injection**

Lotze, Michael T; University of Pittsburgh, Pittsburgh, Pennsylvania; *IL-12 Gene Therapy Using Direct Injection of Tumor with Genetically Engineered Autologous Fibroblasts.*

\*RAC Recommends Approval: 6-10-94/NIH Approval: 2-10-95  
Complete: January 2000

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**9408-082 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Subcutaneous Injection**

Simons, Jonathan; Johns Hopkins Oncology Center, Baltimore, Maryland; *Phase I/II Study of Autologous Human GM-CSF Gene Transduced Prostate Cancer Vaccines in Patients with Metastatic Prostate Carcinoma.*

NIH/ORDA Approval: 8-3-94 (Accelerated Review)  
Study closed, long-term follow-up continues: 7-16-01

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**9409-083 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)**

Zeitlin, Pamela L.; Johns Hopkins Childrens Center, Baltimore, Maryland and Flotte, Terence R., University of Florida, Gainesville, Florida; *A Phase I Study of an Adeno-associated Virus-CFTR Gene Vector in Adult CF Patients with Mild Lung Disease*. Sponsor: Targeted Genetics Corporation

\*RAC Recommends Approval: 9-12-94/NIH Approval: 11-15-94

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**9409-084 (Open) Gene Therapy/Phase I/Cancer/Breast/Antisense /In Vivo/Autologous Tumor Cells/Retrovirus/c-fos Antisense RNA/c-myc Antisense/Intraleural/Intraperitoneal**

Holt, Jeffrey, and Arteaga, Carlos B.; Clinical Research Center at Vanderbilt University Medical Center, Nashville, Tennessee; *Gene Therapy for the Treatment of Metastatic Breast Cancer by In Vivo Infection with Breast-Targeted Retroviral Vectors Expressing Antisense c-fos or Antisense c-myc RNA*.

\*RAC Recommends Approval: 9-12-94/NIH Approval: 1-4-95

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**9409-085 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)/Multiple Dose**

Crystal, Ronald G.; New York Hospital-Cornell Medical Center, New York, New York; *Evaluation of Repeat Administration of a Replication Deficient, Recombinant Adenovirus Containing the Normal Cystic Fibrosis Transmembrane Conductance Regulator cDNA to the Airways of Individuals with Cystic Fibrosis*.

\*RAC Recommends Approval: 9-12-94/NIH Approval: 11-30-94

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**9409-086 (Closed) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Avectin™/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection**

Lyerly, H. Kim; Duke University Medical Center, Durham, North Carolina; *A Pilot Study of Autologous Human Interleukin-2 Gene Modified Tumor Cells in Patients with Refractory or Recurrent Metastatic Breast Cancer*.

\*RAC Recommends Approval: 9-12-94/NIH Approval: 10-25-94

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**9409-087 (Closed) Gene Therapy/Phase I/Monogenic Disease/Hunter Syndrome/In Vitro/Autologous Peripheral Blood Cells/Retrovirus/Iduronate-2-Sulfatase cDNA/Intravenous**

Whitley, Chester B.; University of Minnesota, Minneapolis, Minnesota; *Retroviral-Mediated Transfer of the Iduronate-2-Sulfatase Gene into Lymphocytes for Treatment of Mild Hunter Syndrome (Mucopolysaccharidosis Type II)*.

\*RAC Recommends Approval: 9-13-94/NIH Approval: 8-20-95

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**9409-088 (Closed) Gene Therapy/Phase I/Other/Peripheral Artery Disease/In Vivo/Vascular Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor cDNA/Intraarterial/Angioplasty Catheter/Hydrogel Coated Balloon**

Isner, Jeffrey M. and Walsh, Kenneth; St. Elizabeth's Medical Center, Tufts University, Boston, Massachusetts; *Arterial Gene Transfer for Therapeutic Angiogenesis in Patients with Peripheral Artery Disease*.

\*RAC Recommends Approval: 9-13-94/NIH Approval: 11-15-94  
Follow-up has been completed: 11-29-01

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**9409-089 (Closed) Gene Therapy/Phase I/Cancer/Central Nervous System/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Stereotactic Injection**

Eck, Stephen L. and Alavi, Jane B.; University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *Treatment of Advanced CNS Malignancy with the Recombinant Adenovirus H5.020RSVTK: A Phase I Trial*.

\*RAC Recommends Approval: 9-13-94/NIH Approval: 2-2-96

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**9409-090 (Closed) Gene Therapy/Phase I/Cancer/n/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intrapleural**

Albelda, Steven M.; University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *Treatment of Advanced Mesothelioma with the Recombinant Adenovirus H5.010RSVTK: A Phase I Trial.*

\*RAC Recommends Approval: 9-13-94/NIH Approval: 1-4-95

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**9409-091 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Respiratory Epithelial Cells/Adenovirus/Serotype 2/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Respiratory Epithelial Cells/Bronchoscope**

Dorkin, Henry L.; New England Medical Center, Tufts University, Boston, Massachusetts; and Lapey, Allen; Massachusetts General Hospital, Harvard Medical School, Boston, Massachusetts; *Adenovirus Mediated Gene Transfer for Cystic Fibrosis: Safety of Single Administration in the Lung (lobar instillation).* Sponsor: Genzyme Corporation

NIH/ORDA Approval: 10-5-94 (Accelerated Review)  
Protocol ended in December 1997

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**9411-092 (Closed) Gene Marking/Cancer/Lymphoma/Breast/In Vitro/CD34+ Autologous Bone Marrow Cells/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Douer, Dan; University of Southern California; Kenneth Norris Comprehensive Cancer Center and Hospital, Los Angeles, California; *High Dose Chemotherapy and Autologous Bone Marrow plus Peripheral Blood Stem Cell Transplantation for Patients with Lymphoma or Metastatic Breast Cancer: Use of Marker Genes to Investigate the Biology of Hematopoietic Reconstitution in Adults.*

NIH/ORDA Approval: 11-18-94 (Accelerated Review)  
Notification that trial has been closed: 6-13-01

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**9411-093 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Subcutaneous Injection**

Dranoff, Glen; Dana Farber Cancer Institute, Boston, Massachusetts; *A Phase I Study of Vaccination with Autologous, Irradiated Melanoma Cells Engineered to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor.*

NIH/ORDA Approval: 11-23-94 (Accelerated Review)  
Study closed, long-term follow-up continues: 7-16-01

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**9412-094 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Respiratory Epithelial Cells/Adenovirus/Serotype 2/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Respiratory Epithelial Cells/Aerosol Administration**

Dorkin, Henry L.; New England Medical Center, Tufts University, Boston, Massachusetts; and Lapey, Allen; Massachusetts General Hospital, Harvard Medical School, Boston, Massachusetts; *Adenovirus Mediated Gene Transfer for Cystic Fibrosis: Safety of a Single Administration in the Lung (aerosol administration).* Sponsor: Genzyme Corporation

\*RAC Recommends Approval: 12-1-94/NIH Approval: 7-24-95  
Protocol ended in December 1997

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**9412-095 (Closed) Gene Therapy/Phase I/Solid Tumors/Lymphoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1102/Cytokine/Interleukin-2 cDNA/Intratumoral/Direct Injection**

Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; and Rinehart, John; Scott and White Clinic; Temple Texas. *Phase I Trial of Interleukin-2 Plasmid DNA/DMRIE/DOPE Lipid Complex as an Immunotherapeutic Agent in Solid Malignant Tumors or Lymphomas by Direct Gene Transfer.* Sponsor: Vical, Incorporated

\*RAC Recommends Approval: 12-1-94/NIH Approval: 3-2-95

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**9412-096 (Closed) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral/Bronchoscope**

Clayman, Gary; M.D. Anderson Cancer Center, Houston, Texas; *Clinical Protocol for Modification of Tumor Suppressor Gene Expression in Head and Neck Squamous Cell Carcinoma (HNSCC) with an Adenovirus Vector Expressing Wild-type p53.*

\*RAC Recommends Approval: 12-2-94 and 9-11-95/NIH Approval: 9-21-95  
Closed: July 1997

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**9412-097 (Open) Gene Therapy/Phase I/Cancer/Colon/Hepatic Metastases/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intrahepatic/Hepatic Artery/Bolus Infusion**

Venook, Alan and Warren, Robert; Moffitt-Long Hospital of the University of California, San Francisco Medical Center; *Gene Therapy of Primary and Metastatic Malignant Tumors of the Liver Using ACN53 Via Hepatic Artery Infusion: A Phase I Study*. Sponsor: Schering Plough Corporation (formerly Canji)

RAC Recommends Approval Contingent Upon Meeting Stipulations: 12-2-94

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**9412-098 (Open) Gene Therapy/Phase I/Cancer/Central Nervous System Malignancies/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intra- tumoral/Stereotactic Injection**

Grossman, Robert and Woo, Savio; The Methodist Hospital, Houston, Texas; *Phase I Study of Adenoviral Vector Delivery of the HSV-TK Gene and the Intravenous Administration of Ganciclovir in Adults with Malignant Tumors of the Central Nervous System*.

\*RAC Recommends Approval: 12-2-94/NIH Approval: 2-2-96

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**9502-099 (Closed) Gene Therapy/Phase I/Cancer/Astrocytoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Stereotactic Injection**

Fetell, Michael; Columbia Presbyterian Medical Center, New York, New York; Warnick, Ronald; University of Cincinnati, Cincinnati, OH; Yung, W.K. Alfred; M.D. Anderson Cancer Center, Houston, Texas; Maria, Bernard L.; University of Florida, Gainesville, Florida; Shaffrey, Mark; University of Virginia Health Sciences Center, Charlottesville, Virginia; Ram, Zvi; Chaim Sheba Medical Center, Tel Aviv University Sackler School of Medicine, Tel Hashomer, Israel; Prados, Michael; University of California, San Francisco, California; and Grossman, Stuart; Johns Hopkins University Hospital Oncology Center; Baltimore, Maryland; *Stereotactic Injection of Herpes Simplex Thymidine Kinase Vector Producer Cells (PA317/G1TkSvNa.7) and Intravenous Ganciclovir for the Treatment of Recurrent Malignant Glioma*. Sponsor: Genetic Therapy, Inc./Novartis

NIH/ORDA Approval: 2-10-95 (Accelerated Review)  
Closed: April 1997

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**9503-100 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intraperitoneal/Catheter**

Link, Charles; Human Gene Therapy Research Institute; and Moorman, Donald; Iowa Methodist Medical Center, Des Moines, Iowa; *A Phase I Trial of In Vivo Gene Therapy with Herpes Simplex Thymidine Kinase/Ganciclovir System for the Treatment of Refractory or Recurrent Ovarian Cancer*.

\*RAC Recommends Approval: 3-6-95/NIH Approval: 7-27-95

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**9503-101 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-7 cDNA/Hygromycin Phosphotransferase/Herpes Simplex Virus Thymidine Kinase cDNA/Subcutaneous Injection**

Economou, James; Glaspy, John; and McBride, William; University of California, Los Angeles, California; *A Phase I Testing of Genetically Engineered Interleukin-7 Melanoma Vaccines*.

\*RAC Recommends Approval: 3-6-95/NIH Approval: 8-20-95  
Closed: 3-97

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**9503-102 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/HLA-Matched Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-2 cDNA/Gamma Interferon cDNA/Subcutaneous Injection**

Gansbacher, Bernd; Memorial Sloan Kettering Cancer Center, New York, New York; *Phase I/II Study of Immunization with MHC Class I Matched Allogeneic Human Prostatic Carcinoma Cells Engineered to Secrete Interleukin-2 and Interferon-γ*.

RAC Recommends Approval Contingent Upon Meeting Stipulations: 3-6-95

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**9503-103 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/Antisense TAR/Transdominant Rev/Intravenous**

Tavel, Jorge; National Institutes of Health, Bethesda, Maryland; *Gene Therapy for AIDS using Retroviral Mediated Gene Transfer to Deliver HIV-1 Antisense TAR and Transdominant Rev Protein Genes to Syngeneic Lymphocytes in HIV Infected Identical Twins*.

\*RAC Recommends Approval: 3-7-95/NIH Approval: 4-1-95  
Closed to new enrollment. Individuals will be followed, long-term, in a new protocol (that does not involve administration of recombinant DNA): 1-17-02

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**9503-104 (Closed) Gene Therapy/Phase I/Monogenic Disease/Chronic Granulomatous Disease  
In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/p47phox/Intravenous**

Malech, Harry; National Institutes of Health, Bethesda, Maryland; *Gene Therapy Approach for Chronic Granulomatous Disease*.

\*RAC Recommends Approval: 3-7-95/NIH Approval: 4-15-95  
Closed to enrollment: 4-12-04

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**9503-105 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Muscle  
Cells/Retrovirus/HIV-1IIIB Envelope Protein/Intramuscular Injection**

Parenti, David; George Washington University Medical Center, Washington, D.C.; Haubrich, Richard; University of California San Diego Treatment Center, San Diego, California; Frame, Peter; University of Cincinnati AIDS Treatment Center, Cincinnati, Ohio; Powderly, William; Washington University AIDS Clinical Trials Unit, St. Louis, Missouri; and Loveless, Mark; Oregon Health Sciences University, Portland, Oregon; *A Repeat Dose Safety and Efficacy Study of HIV-IT(V) in HIV-1 Infected Subjects with Greater Than or Equal to 100 CD4+ T Cells and No AIDS Defining Symptoms*.

NIH/ORDA Approval: 3-11-95 (Accelerated Review)  
Notification that trial has been completed, IND is inactive: 5-22-00

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**9506-106 (Open) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/Autologous G-CSF and ATA-C Mobilized Bone Marrow  
Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Verfaillie, Catherine; University of Minnesota, Minneapolis, Minnesota; *Autologous Marrow Transplantation for Chronic Myelogenous Leukemia Using Stem Cells Obtained After In Vivo Chemotherapy Cytokine Priming*.

NIH/ORDA Approval: 5-5-95

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**9506-107 (Closed) Gene Therapy/Phase I/Cancer/Multiple Myeloma/Pro-Drug/In Vitro/Allogeneic T Lymphocytes/Retrovirus/Herpes Simplex  
Thymidine Kinase/Ganciclovir/Intravenous**

van Rhee, Frits and Barlogie, Bart; University of Arkansas for Medical Sciences, Little Rock, Arkansas; *Thymidine Kinase (TK) Transduced Donor Leukocyte Infusions as a Treatment for Patients with Relapsed or Persistent Multiple Myeloma after T-cell Depleted Allogeneic Bone Marrow Transplant*.  
Sponsor: Genetic Therapy, Inc./Novartis

\*RAC Recommends Approval: 6-9-95/NIH Approval: 7-27-95  
Closed: 5-30-03

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**9506-108 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally  
Irradiated/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin  
cDNA/Subcutaneous Injection**

Fox, Bernard A. and Urba, Walter J.; Earle A. Chiles Research Institute, Providence Medical Center, Portland, Oregon; *Adoptive Cellular Therapy of Cancer Combining Direct HA-B7/β-2 Microglobulin Gene Transfer with Autologous Tumor Vaccination for the Generation of Vaccine-Primed Anti-CD3 Activated Lymphocytes*.

\*RAC Recommends Approval: 6-9-95/NIH Approval: 9-30-95

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**9506-109 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vitro/Anti-CD3 Stimulated Autologous Peripheral Blood  
Lymphocytes/Retrovirus/Antibody/MOV-gamma (Reactive with Folate Binding Protein)/Intravenous/Intraperitoneal**

Hwu, Patrick; National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Advanced Epithelial Ovarian Cancer using Anti-CD3 Stimulated Peripheral Blood Lymphocytes Transduced with a Gene Encoding a Chimeric T-cell Receptor Reactive with Folate Binding Protein*.

RAC Recommends Approval Contingent Upon Meeting Stipulations: 6-9-95

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**9506-110 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic  
Liposome Complex/DDAB-DOPE/Cytokine/Interleukin-2 cDNA/Intradermal Injection**

Berchuck, Andres and Lyerly, H. Kim; Duke University Medical Center, Durham, North Carolina; *A Phase I Study of Autologous Human Interleukin-2 (IL-2) Gene Modified Tumor Cells in Patients with Refractory Metastatic Ovarian Cancer*.

\*RAC Recommends Approval: 6-10-95/NIH Approval: 9-30-95

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**9506-111 (Closed) Gene Therapy/Phase I/Monogenic Disease/Purine Nucleoside Phosphorylase Deficiency/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/Purine Nucleoside Phosphorylase cDNA/Intravenous**

Mclvor, R. Scott; Institute of Human Genetics, University of Minnesota, Minneapolis, Minnesota; *Gene Therapy for Purine Nucleoside Phosphorylase Deficiency*.

\*RAC Recommends Approval: 6-9-95/NIH Approval: 7-27-95

Closed: 9-24-00 No individuals enrolled, IND not submitted

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**9506-112 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Single Chain Antibody Gene/In Vitro/CD4+ Autologous Peripheral Blood Lymphocytes/Retrovirus/sFv105 Anti-HIV-1 Envelope Protein(gp160)Gene/Intravenous**

Marasco, Wayne A.; Dana Farber Cancer Institute, Boston, Massachusetts; *Intracellular Antibodies Against HIV-1 Envelope Protein for AIDS Gene Therapy*.

\*RAC Recommends Approval: 6-9-95/NIH Approval: 7-27-95

Never activated: 4-21-04

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**9504-113 (Closed) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus-1/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1IIIB Envelope Protein/Intramuscular Injection**

Conant, Marcus, Conant Medical Group; Lang, William, ViRx, Inc.; and Merritt, James, Viagene, Inc., San Francisco, California; *A Randomized, Double Blinded, Phase I/II Dosing Study to Evaluate the Safety and Optimal CTL Inducing Dose of HIV-IT(V) in Pre-Selected HIV-1 Infected Subjects*.

\*RAC Recommends Approval: NA/NIH Approval: NA (Non-NIH funded institution)

FDA Approval: 5-6-94

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**9507-114 (Closed) Gene Therapy/Phase I-II/Monogenic Disease/Cystic Fibrosis/In Vivo/Maxillary Sinus Epithelial Cells/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Maxillary Sinus Administration**

Gardner, Phyllis; Stanford University School of Medicine, Stanford, California; *A Phase I/II Study of tg-CF for the Treatment of Chronic Sinusitis in Patients with Cystic Fibrosis*. Sponsor: Targeted Genetics Corporation

Sole FDA Review Recommended by NIH/ORDA: 7-11-95

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**9508-115 (Closed) Gene Therapy/Phase II/Cancer/Metastatic Malignancies(Breast Adenocarcinoma, Renal Cell Carcinoma, Melanoma, Colorectal Adenocarcinoma, non-Hodgkin's Lymphoma)/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL 1005/HLA-B7/Beta-2 Microglobulin cDNA/Direct Intratumoral Injection**

Chang, Alfred E.; University of Michigan Medical Center, Ann Arbor, Michigan; Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; Vogelzang, Nicholas; University of Chicago Medical Center, Chicago, Illinois; Levy, Ronald; Stanford University Medical Center, Palo Alto, California; Redman, Bruce; Wayne State University School of Medicine; Detroit, Michigan; Figlin, Robert; University of California Medical Center, Los Angeles, California; Rubin, Joseph; Mayo Foundation for Medical Evaluation and Research, Rochester, Minnesota; Rinehart, John J.; Scott and White Hospital, Texas A & M University, Temple Texas; Doroshow, James H.; City of Hope National Medical Center, Duarte, California; Klasa, Richard; British Columbia Cancer Agency, Vancouver, British Columbia; Sobol, Robert; Sidney Kimmel Cancer Center, San Diego, California; *Phase II Study of Immunotherapy of Metastatic Cancer by Direct Gene Transfer*. Sponsor: Vical, Incorporated

Sole FDA Review Recommended by NIH/ORDA: 8-2-95

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**9508-116 (Open) Gene Therapy/Phase I/Cancer/Glioma/Immunotherapy/In Vitro/Autologous Tumor (Glioma) Cells/Non-Irradiated/Retrovirus/Cytokine/Interleukin-4 cDNA/Subcutaneous Injection**

Pollack, Ian; Okada, Hideho; and Lotze, Michael T.; University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; *Gene Therapy of Malignant Gliomas: A Phase I Study of IL-4 Gene -Modified Autologous Tumor to Elicit an Immune Response*.

Sole FDA Review Recommended by NIH/ORDA: 8-7-95

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**9508-117 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus-1/Replication Inhibition/In Vitro/Autologous CD34+ Peripheral Blood Cells/Retrovirus/Hammerhead Ribozyme/Intravenous**

Mitsuyasu, Ronald; University of California Los Angeles, California; *A Phase I Trial of Autologous CD34+ Hematopoietic Progenitor Cells Transduced with an Anti-HIV-1 Ribozyme*.

Sole FDA Review Recommended by NIH/ORDA: 8-7-95

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**9508-118 (Open) Gene Therapy/Phase I/Other/Restenosis/In Vivo/Vascular Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor cDNA/Intraarterial/Angioplasty Catheter/Hydrogel Coated Balloon**

Losordo, Douglas W.; St. Elizabeth's Medical Center, Tufts University School of Medicine, Boston, Massachusetts; *Accelerated Re-endothelialization and Reduced Neointimal Thickening Following Catheter Transfer of phVEGF165.*

Sole FDA Review Recommended by NIH/ORDA: 8-7-95

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**9508-119 (Open) Gene Therapy/Phase I/Human Immunodeficiency Virus-1/In Vitro/CD8+ Allogeneic Cytotoxic T Lymphocytes/CD8+ Syngeneic Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase/Herpes Simplex Virus Thymidine Kinase cDNA/Retrovirus/Intravenous**

Riddell, Stanley R.; Fred Hutchinson Cancer Research Center, Seattle, Washington; *Phase I Study to Evaluate the Safety of Cellular Adoptive Immunotherapy using Autologous Unmodified and Genetically Modified CD8+ HIV-Specific T Cells in HIV Seropositive Individuals.* Sponsor: Targeted Genetics Corporation

Sole FDA Review Recommended by NIH/ORDA: 8-7-95

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**9508-120 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Used to Derive Tumor Infiltrating Lymphocytes/HLA-B7 cDNA/Intravenous**

Chang, Alfred E. and Nabel, Gary J.; University of Michigan Medical Center, Ann Arbor, Michigan; *Phase I Study of Tumor-Infiltrating Lymphocytes Derived from In Vivo HLA-B7 Gene Modified Tumors in the Adoptive Immunotherapy of Melanoma.*

Sole FDA Review Recommended by NIH/ORDA: 8-14-95

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**9508-121 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vivo/Autologous Tumor Cells/HLA B7 cDNA/Intratumoral/Concurrent Interleukin-2 Therapy**

Figlin, Robert A.; University of California Los Angeles Medical Center, Los Angeles, California; *Phase I Study of HLA-B7 Plasmid DNA/DMRIE/DOPE Lipid Complex as an Immunotherapeutic Agent in Renal Cell Carcinoma by Direct Gene Transfer with Concurrent Low Dose Bolus IL-2 Protein Therapy.* Sponsor: Vical, Incorporated

Sole FDA Review Recommended by NIH/ORDA: 8-14-95

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**9508-122 (Closed) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies (type of cancer not specified)/Immunotherapy/In Vivo/Autologous Muscle Cells/Canarypox Virus/Carcinoembryonic Antigen cDNA/Intramuscular Injection**

Hawkins, Michael J. and Marshall, John L.; Georgetown University Medical Center, Washington, D.C.; *A Study of Recombinant ALVAC Virus that Expresses Carcinoembryonic Antigen in Patients with Advanced Cancers.*

Sole FDA Review Recommended by NIH/ORDA 8-14-95  
Closed: 2-97

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**9509-123 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Antisense/In Vivo/Autologous Tumor Cells/Retrovirus/Antisense c-myc RNA/Intraprostate Injection**

Steiner, Mitchell S., Clinical Research Center at Vanderbilt University Medical Center, Nashville, Tennessee; and Holt, Jeffrey T., Vanderbilt University School of Medicine, Nashville, Tennessee; *Gene Therapy for the Treatment of Advanced Prostate Cancer by In Vivo Transduction with Prostate-Targeted Retroviral Vectors Expressing Antisense c-myc RNA.*

\*RAC Recommends Approval: 9-11-95/NIH Approval: 9-30-95

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**9509-124 (Closed) Gene Therapy/Phase I/Cancer/Ovarian and Extraovarian/Anti-erbB-2 Single Chain Antibody Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Anti-erbB-2 (oncoprotein/extracellular domain) Single-chain Antibody Gene/Intraperitoneal Injection**

Curiel, David T. and Alvarez, Ronald D.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Study of Recombinant Adenovirus Vector-Mediated Delivery of an Anti-erbB-2 Single Chain (sFv) Antibody Gene for Previously Treated Ovarian and Extraovarian Cancer Patients.*

RAC Recommends Approval Contingent Upon Meeting Stipulations: 9-11-95  
Completed: 4-13-99

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**9509-125 (Closed) Gene Therapy/Phase I/Cancer/Colon Carcinoma (Hepatic Metastases)/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/E. coli Cytosine Deaminase cDNA/Intratumoral (Hepatic) Injection/Combined with Oral 5-Fluorocytosine**

Crystal, Ronald, G.; Hershowitz, Edward; and Lieberman, Michael; New York Hospital-Cornell Medical Center, New York, New York; *A Phase I Study of Direct Administration of a Replication-Deficient Adenovirus Vector Containing the E. coli Cytosine Deaminase Gene to Metastatic Colon Carcinoma of the Liver in Association with the Oral Administration of the Pro-Drug 5-Fluorocytosine.*

\*RAC Recommends Approval: 9-11-95/NIH Approval: 9-30-95  
Notification that IND has been withdrawn: 2-2-00

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**9509-126 (Open) Gene Therapy/Phase I/Cancer/Prostate Adenocarcinoma/Immunotherapy/In Vivo/Vaccination/Vaccinia Virus/Prostate Specific Antigen/Intradermal Injection**

Chen, A.P.; National Naval Medical Center, Bethesda, Maryland; *A Phase I Study of Recombinant Vaccinia that Expresses Prostate Specific Antigen in Adult Patients with Adenocarcinoma of the Prostate.*

Sole FDA Review Recommended by NIH/ORDA: 9-22-95

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**9509-127 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cationic Liposome Complex/DOPE/Cystic Fibrosis Transmembrane Conductance Regulator cDNA; Intranasal Administration**

Welsh, Michael J. and Zabner, Joseph; Howard Hughes Medical Institute, University of Iowa College of Medicine, Iowa City, Iowa; *Cationic Lipid Mediated Gene Transfer of CFTR: Safety of a Single Administration to the Nasal Epithelia.* Sponsor: Genzyme Corporation

Sole FDA Review Recommended by NIH/ORDA: 9-26-95  
Completed: 12-97

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**9510-128 (Closed) Gene Therapy/Phase I/Cancer/Gastrointestinal Tract, Breast, or Lung Adenocarcinoma (CEA-Expressing Malignancies)/Immunotherapy/In Vivo/Vaccination/Vaccinia Virus/Carcinoembryonic Antigen/Intradermal Injection in Combination with Subcutaneous Peptide Challenge**

Cole, David J.; Medical University of South Carolina, Charleston, South Carolina; *Phase I Study of Recombinant CEA Vaccinia Virus Vaccine with Post Vaccination CEA Peptide Challenge.*

Sole FDA Review Recommended by NIH/ORDA: 10-16-95  
Closed: 9-99

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**9510-129 (Open) Gene Marking/Cancer/EBV-Positive Hodgkin Disease/In Vitro/EBV-Specific Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Roskrow, Marie; Hudson, Melissa; Rooney, Cliona; Heslop, Helen; and Brenner, Malcolm; St. Jude Children's Research Hospital, Memphis, Tennessee; *Administration of Neomycin Resistance Gene Marked EBV Specific Cytotoxic T Lymphocytes as Therapy for Patients Receiving a Bone Marrow Transplant for Relapsed EBV-Positive Hodgkin Disease.*

Sole FDA Review Recommended by NIH/ORDA: 10-17-95

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**9510-130 (Open) Gene Marking/Cancer/EBV-Positive Hodgkin Disease/In Vitro/EBV-Specific Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Administration**

Roskrow, Marie; Hudson, Melissa; Rooney, Cliona; Heslop, Helen; and Brenner, Malcolm; St. Jude Children's Research Hospital, Memphis, Tennessee; *Administration of Neomycin Resistance Gene Marked EBV Specific Cytotoxic T Lymphocytes to Patients with Relapsed EBV-Positive Hodgkin Disease.*

Sole FDA Review Recommended by NIH/ORDA: 10-17-95

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**9510-131 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD8+ T Cells/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous**

Connick, Elizabeth; University of Colorado Health Sciences Center, Denver, Colorado; and Deeks, Steven G.; University of California, San Francisco General Hospital, San Francisco, California; *A Randomized, Controlled, Phase II Study of the Activity and Safety of Autologous CD4-Zeta Gene-Modified T Cells in HIV-Infected Patients.* Sponsor: Cell Genesys, Inc.

Sole FDA Review Recommended by NIH/ORDA: 10-17-95  
Closed 8-6-97 (No longer enrolling patients)

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**9510-132 (Open) Gene Therapy/Phase I/Cancer/Locally Advanced or Metastatic Prostate/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Cytokine/Interleukin-2 cDNA/Intradermal Injection**

Paulson, David; and Lyerly, H. Kim; Duke University Medical Center, Durham, North Carolina; *A Phase I Study of Autologous Human Interleukin-2 (IL-2) Gene Modified Tumor Cells in Patients with locally Advanced or Metastatic Prostate Cancer.*

Sole FDA Review Recommended by NIH/ORDA: 10-19-95

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**9511-133 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous Tumor Cells (Non-irradiated)/Type 5 Adenovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection**

Brenner, Malcolm K.; Dilloo, Dagmar; and Bowman, Laura; St. Jude Children's Research Hospital, Memphis, Tennessee; *Phase I Study of Cytokine Gene Modified Autologous Neuroblastoma Cells for Treatment of Relapsed/Refractory Neuroblastoma Using an Adenoviral Vector.*

Sole FDA Review Recommended by NIH/ORDA: 11-1-95

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**9511-134 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/Autologous CD4+ T Cells/Retrovirus/Neomycin Phosphotransferase Gene/PolyTAR Decoy Gene/RRE-polyTAR Decoy Gene**

Greenberg, Philip D.; Fred Hutchinson Cancer Research Center, University of Washington Medical Center, Seattle, Washington; *Phase I Study to Evaluate the Safety and In Vivo Persistence of Adoptively Transferred Autologous CD4+ T Cells Genetically Modified to Resist HIV Replication.*

Sole FDA Review Recommended by NIH/ORDA: 11-1-95

Trial is closed to new accrual; follow-up will continue: 03-19-01

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**9511-135 (Closed) Gene Therapy/Phase I/Cancer/Ovarian and Extraovarian Cancer/Single Chain Antibody/In Vivo/Autologous Tumor Cells/Adenovirus/Herpes Simplex Thymidine Kinase Gene/Intraperitoneal Injection/Combined with Intravenous Ganciclovir Administration**

Alvarez, Ronald D. and Curiel, David T.; University of Alabama Comprehensive Cancer Center, Birmingham, Alabama; *A Phase I Study of Recombinant Adenovirus Vector-Mediated Intraperitoneal Delivery of Herpes Simplex Virus Thymidine Kinase (HSV-TK) Gene and Intravenous Ganciclovir for Previously Treated Ovarian and Extraovarian Cancer Patients.*

Sole FDA Review Recommended by NIH/ORDA: 11-1-95  
Completed: 5-04-00

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**9511-136 (Open) Gene Therapy/Phase I/Cancer/Metastatic Melanoma/Immunotherapy In Vitro/Autologous CD8+ Tyrosinase-Specific T Cells/Retrovirus/Hygromycin Phosphotransferase/Intravenous Administration**

Yee, Cassian and Greenberg, Philip D.; Fred Hutchinson Cancer Research Center, University of Washington Medical Center, Seattle, Washington; *Phase I Study to Evaluate the Safety of Cellular Adoptive Immunotherapy Using Autologous Unmodified and Genetically Modified CD8+ Tyrosinase-Specific T Cells in Patients with Metastatic Melanoma.*

Sole FDA Review Recommended by NIH/ORDA: 11-1-95

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**9512-137 (Open) Gene Therapy/Phase I/Cancer/Ovarian,Breast/Oncogene Regulation/HER-2/neu/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal, Intrapleural Administration**

Hortobagyi, Gabriel N.; Lopez-Berstein, Gabriel; and Hung, Mien-Chien; M.D. Anderson Cancer Center, Houston, Texas; Kilbourn, Robert, Rush-Presbyterian/St. Luke's Medical Center, Chicago, Illinois; Weiden, Paul; Virginia Mason Medical Center, Seattle, Washington; *Phase I Study of E1A Gene Therapy for Patients with Metastatic Breast or Ovarian Cancer that Overexpresses Her-2/neu.* Sponsor: Targeted Genetics Corporation

\*RAC Recommends Approval: 12-4-95/NIH Approval: 2-2-96

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**9512-138 (Closed) Gene Therapy/Phase I/Cancer/Malignant Glioma/Antisense/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Plasmid DNA--Electroporation/TGF- $\beta$ 2/Subcutaneous Injection**

Black, Keith L.; and Fakhrai, Habib; University of California, Los Angeles, School of Medicine, Los Angeles, California; *A Phase I Study of the Safety of Injecting Malignant Glioma Patients with Irradiated TGF- $\beta$ 2 Antisense Gene Modified Autologous Tumor Cells.*

\*RAC Recommends Approval: 12-4-95/NIH Approval: 4-2-96  
Closed: 5-22-03

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**9512-139 (Open) Gene Therapy/Phase I/Monogenic Disease/Partial Ornithine Transcarbamylase (OTC) Deficiency/In Vivo/Autologous Peripheral Blood Cells/Adenovirus/Type 5 (E2a Temperature-Sensitive Mutant)/Ornithine Transcarbamylase cDNA/Intravenous**

Batshaw, Mark; Institute for Human Gene Therapy, University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *A Phase I Study of Adenoviral Vector Mediated Gene Transfer to Liver in Adults with Partial Ornithine Transcarbamylase Deficiency.*

RAC Recommends Approval Contingent Upon Meeting Stipulations: 12-4-95

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**9512-140 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Type 2/MART-1 Melanoma Antigen/Subcutaneous Injection/Immunization**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Phase I Trial in Patients with Metastatic Melanoma of Immunization with a Recombinant Adenovirus Encoding the MART-1 Melanoma Antigen.*

Sole FDA Review Recommended by NIH/ORDA: 12-1-95

Closed: 9-17-99, follow-up continuing

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**9512-141 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus-1/Replication Inhibition/In Vitro/Autologous CD4+ Peripheral Blood Lymphocytes/Retrovirus/Anti-Rev SFv/Intravenous**

Pomerantz, Roger J; Jefferson Medical College, Thomas Jefferson University, Philadelphia, Pennsylvania; *Intracellular Immunization Against HIV-1 Infection Using an Anti-Rev Single Chain Variable Fragment (SFv).*

Sole FDA Review Recommended by NIH/ORDA: 12-13-95

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**9512-142 (Open) Gene Therapy/Phase I/Gene Therapy/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL 1005/HLA-B7/Beta-2 Microglobulin cDNA/Direct Intratumoral Injection**

Gluckman, Jack L.; University of Cincinnati Medical Center, Cincinnati, Ohio; *Alloectin-7 in the Treatment of Squamous Cell Carcinoma of the Head and Neck.*

Sole FDA Review Recommended by NIH/ORDA: 12-15-95

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**9601-143 (Closed) Gene Therapy/Phase I/Cancer/Breast/Chemoprotection/In Vitro/Autologous CD34+ Peripheral Blood Lymphocytes/Retrovirus/Multi-Drug Resistance-1 cDNA/Neomycin Phosphotransferase cDNA/Intravenous**

Cowan, Kenneth H.; National Institutes of Health, Bethesda, Maryland; *Antimetabolite Induction, High-Dose Alkylating Agent Consolidation, and Retroviral Transduction of the MDR1 Gene Into Peripheral Blood Progenitor Cells Followed by Intensification Therapy with Sequential Paclitaxel and Doxorubicin for Stage 4 Breast Cancer.*

Sole FDA Review Recommended by NIH/ORDA: 1-26-96

Closed: 6-14-00

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**9601-144 (Open) Gene Therapy/Phase I/Cancer/Prostate/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Intra-prostatic Tumor Injection**

Scardino, Peter T.; Thompson, Timothy C.; and Woo, Savio L.C.; Baylor College of Medicine, Houston, Texas; *Phase I Study of Adenoviral Vector Delivery of the HSV-tk Gene and the Intravenous Administration of Ganciclovir in Men with Local Recurrence of Prostate Cancer after Radiation Therapy.*

Sole FDA Review Recommended by NIH/ORDA: 1-29-96

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**9601-145 (Closed) Gene Therapy/Phase I/Cancer/Bladder/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Retinoblastoma cDNA/Intravesical Catheter Administration**

Small, Eric J. and Carroll, Peter R.; University of California, San Francisco, California; *Gene Therapy of Bladder Cancer Using Recombinant Adenovirus Containing the Retinoblastoma Gene (ACNRB): A Phase IA Study.* Sponsor: Schering Plough Corporation (formerly Canji)

Sole FDA Review Recommended by NIH/ORDA: 1-30-96

Canceled: 4-4-97

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**9602-146 (Open) Gene Therapy/Phase I/Cancer/Hematologic Malignancies Following Allogeneic Bone Marrow Transplant/ProDrug/Elimination of Graft Versus Host Disease/In Vitro/Allogeneic Peripheral Blood Lymphocytes/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous**

Link, Charles J.; Human Gene Therapy Research Institute, Des Moines, Iowa; Burt, Richard K. and Traynor, Ann; Northwestern University School of Medicine, Chicago, Illinois; *Adoptive Immunotherapy for Leukemia: Donor Lymphocytes Transduced with the Herpes Simplex Thymidine Kinase Gene for Remission Induction.*

Sole FDA Review Recommended by NIH/ORDA: 2-8-96

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**9602-147 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD34+ Autologous Bone Marrow Cells/Retrovirus/RRE Decoy Gene, and Retrovirus/Neomycin Phosphotransferase Gene/Intravenous**

Kohn, Donald B.; Childrens Hospital Los Angeles, Los Angeles, California; *Transduction of CD34+ Cells from the Bone Marrow of HIV-1 Infected Children: Comparative Marking by an RRE Decoy and a Neutral Gene.*

Sole FDA Review Recommended by NIH/ORDA: 2-8-96  
Study is in follow-up: 6-22-00

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**9602-148 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection**

O'Malley, Bert W.; Johns Hopkins University, Baltimore, Maryland; *Phase I Study of Adenoviral Vector Delivery of the HSV-tk Gene and the Intravenous Administration of Ganciclovir in Adults with Recurrent or Persistent Head and Neck Cancer.*

Sole FDA Review Recommended by NIH/ORDA: 2-13-96

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**9603-149 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Retrovirus/BRCA-1 Gene/Intraperitoneal Administration (Ultrasound Guided)**

Holt, Jeffrey T.; Clinical Research Center at Vanderbilt University Medical Center, Nashville, Tennessee; *Ovarian Cancer Gene Therapy with BRCA-1.*

Sole FDA Review Recommended by NIH/ORDA: 3-6-96  
Closed: 07-99

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**9603-150 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy In Vivo/Autologous Tumor Cells/HLA B7/cDNA/Intratumoral/Concurrent Interleukin-2 Therapy**

Hersh, Evan M.; Arizona Cancer Center, Tucson, Arizona; and Sondak, Vernon K.; University of Michigan Medical Center, Ann Arbor, Michigan; *Evaluation of Intratumoral Gene Therapy with HLA-B7/DMRIE/DOPE plus Subcutaneous Low Dose IL-2.*

Sole FDA Review Recommended by NIH/ORDA: 3-26-96  
Closed: 3-11-97. Protocol Never Initiated

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**9604-151 (Closed) Gene Therapy/Phase I/ Cancer/Melanoma/Immunotherapy In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 2/GP100 Melanoma Antigen/Subcutaneous or Intramuscular Injection/Concurrent Interleukin-2 Therapy**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Phase I Trial in Patients with Metastatic Melanoma of Immunization with a Recombinant Adenovirus Encoding the GP100 Melanoma Antigen.*

Sole FDA Review Recommended by NIH/ORDA: 4-19-96  
Terminated: 9-17-99

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**9604-152 (Closed) Gene Therapy/Phase I/Inherited Genetic Disorder/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/Correction In Vitro/CD34+ Autologous Umbilical Cord Blood or Bone Marrow/Retrovirus/cDNA for Common  $\gamma$  Chain of Multiple Cytokine Receptors/Intravenous**

Weinberg, Kenneth I.; Childrens Hospital Los Angeles (CHLA) ; Los Angeles, California; *Gene Therapy for X-linked Severe Combined Immune Deficiency using Retroviral Mediated Transduction of the  $\gamma$ c cDNA into CD34+ Cells.*

Sole FDA Review Recommended by NIH/ORDA: 4-24-96  
Study replaced by 0108-494; never initiated: 5-28-03

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**9604-153 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Hammerhead Ribozyme/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Tat and Rev Hammerhead Ribozyme/Intravenous**

Kohn, Donald B.; Childrens Hospital of Los Angeles (CHLA), Los Angeles, California; and Zaia, John A.; City of Hope National Medical Center, Duarte, California; *Transduction of CD34+ Autologous Peripheral Blood Progenitor Cells from HIV-1 Infected Persons: a Phase I Study of Comparative Marking Using a Ribozyme Gene and a Neutral Gene.*

Sole FDA Review Recommended by NIH/ORDA: 4-24-96

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**9605-154 (Closed) Gene Therapy/Phase I/Cancer/Brain Tumors/Pro-Drug/In Vivo/Autologous Tumor Cells/psiCRIP-MFG-S-TK1-67 Cells/Retrovirus/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection**

Harsh IV, Griffith R.; Chiocca, E. Antonio; and Hochberg, Fred H.; Harvard Medical School, Boston, Massachusetts; *Phase I Study of Retroviral-Mediated Incorporation of the HSV Thymidine Kinase Gene and Ganciclovir in Malignant Gliomas.*

Sole FDA Review Recommended by NIH/ORDA: 5-1-96  
Terminated: 6-11-98

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**9605-155 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Cationic Liposome Complex/B7(CD80) cDNA/Retrovirus/Herpes Simplex Thymidine Kinase/Ganciclovir/Intraperitoneal**

Freeman, Scott M.; and Robinson III, William R.; Tulane University School of Medicine, New Orleans, Louisiana; *Tumor Vaccination With HER-2/Neu Using a B7 Expressing Tumor Cell Line Prior To Treatment With HSV-TK Gene-Modified Cells.*

Sole FDA Review Recommended by NIH/ORDA: 5-2-96

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**9608-156 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/B7(CD80) cDNA/Subcutaneous Injection**

Urba, Walter J.; Providence Portland Medical Center, Portland, Oregon; *Phase I Trial Using a CD80-Modified Allogeneic Breast Cancer Line to Vaccinate HLA-A2-Positive Women with Breast Cancer .*

Sole FDA Review Recommended by NIH/ORDA: 8-6-96

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**9608-157 (Closed) Gene Therapy/Phase III of #9303-037/Cancer/Glioblastoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection**

Maria, Bernard; University of Florida, Gainesville, Florida; Gutheil, John; Sharp Healthcare, Sidney Kimmel Cancer Center, San Diego, California; Bucholz, Richard; St. Louis University, St. Louis, Missouri; Olson, Jeffrey; Emory School of Medicine, Winship Cancer Center, Atlanta, Georgia; Lillehei, Kevin; University of Colorado, Denver, Colorado; Van Gilder, John; University of Iowa College of Medicine, Iowa City, Iowa; Nemunaitis, John; Texas Oncology P.A., Baylor University Medical Center, Dallas, Texas; Origitano, Thomas; Loyola University Medical Center, Maywood, Illinois; Warnick, Ronald; University of Cincinnati Medical Center, The Christ Hospital, Good Samaritan Hospital, Jewish Hospital of Cincinnati, Veterans Affairs Medical Center, Cincinnati, Ohio; Weber, Friederich Dr. med.; Heinrich Heine Universität, Düsseldorf, Germany; Rainov, Nikolai, PD Dr. med.; Martin Luther Universität, Halle, Germany; Cloughesy, Timothy; UCLA Department of Neurology, Reed Neurological Research Center, Boywer Oncology Clinic, Los Angeles, California; Markert, James; University of Alabama at Birmingham, Birmingham, Alabama; Matti Vapalahti, Kuopio University Hospital, Kuopio, Finland; Yasuhiro Yonekawa, University Hospital, Zurich, Switzerland; Nanno Harrie Mulder, Academic Hospital Groningen, Groningen, The Netherlands; Susanne Osante, Academic Hospital Leiden, Leiden, The Netherlands; Fetell, Michael; Columbia-Presbyterian Medical Center Neurological Institute, New York, New York; Schramm, Johannes; Prof. Dr. med., Univ. Klinikum Neurochirurgische Klinik, Bonn, Germany; Westphal, Manfred, PD Dr. med.; Klinikum Eppendorf Neurochirurgie/Univ. Martinstr. 52, Hamburg, Germany; Tonn, Jorg-Christian, PD Dr. med.; u. Poliklinik/Univ. Kliniken, Würzburg, Germany; Moumdjian, Robert, Dr.; Hospital Notre-Dame, Montreal, Quebec, Canada; Shaffrey, Mark; University of Virginia, Charlottesville, Virginia; Asher, Anthony; Presbyterian Hospital, Cancer Center, Charlotte, North Carolina; Epstein, Mel; Brown University, Providence, Rhode Island; Schmidt-Schackert, Frau.Prof. Dr. med.; Gabriele, Univ.-Klin. Kar-G. Carus, Klinik f. Neurochirurgie, Dresden, Germany; Mendez, Ivar; Victoria General Hospital, Halifax, Nova Scotia, Canada; Bernstein, Mark, The Toronto Hospital, Toronto, Ontario, Canada; Quigley, Mathew, Allegheny University of Health Sciences, Pittsburgh, Pennsylvania; Payner, Troy; Indianapolis Surgical Group, Indianapolis, Indiana; Kulvik, Martti; Helsinki University Central Hospital, Helsinki, Finland; Seiler, Rolf W.; University Hospital, Bern, Switzerland; Weiss, Martin Harvey; University of Southern California, Department of Neurosurgery, Los Angeles, California; Fick, James R.; Medical College of Georgia, Department of Surgery, Augusta, Georgia; Leblanc, Richard; Montreal Neurological Institute, Montreal, Quebec, Canada; Buchfelder, Michael; Neurochirurgische Klinik mit Poliklinik der Universität Erlangen-Nürnberg, Erlangen, Germany; Brotschi, Jacques; Hôpital Erasme, Neurosurgery, Cliniques Universitaires de Bruxelles, Bruxelles, Belgium; Astrup, Jens; Århus Kommunehospital, Århus C, Denmark; Henriksson, Roger; University Hospital, Umeå, Sweden; Maciunas, Robert J.; Vanderbilt University Medical Center, Nashville, Tennessee; Ram, Zvi; The Chaim Sheba Medical Center; Tel-Hashomer, Israel; Andrews, David; Thomas Jefferson University Hospital, Philadelphia, Pennsylvania; Verlooy, Jan; University Hospital Antwerp; Antwerp, Belgium; Stockhammer, Gunther; Universitätsklinik für Neurologie, Innsbruck, Austria; Favrot, Marie; Centre Leon Berard, Lyon, France; and Finocchiaro, Gaetano; Unita Neuroncologia Molecolare e Terapia Genica, Istituto Nazionale Neurologico Carlo Besta, Milano, Italy; *Prospective, Open-Label, Parallel-Group, Randomized Multicenter Trial Comparing the Efficacy of Surgery, Radiation, and Injection of Murine Cells Producing Herpes Simplex Thymidine Kinase Vector Followed by Intravenous Ganciclovir Against the Efficacy of Surgery and Radiation in the Treatment of Newly Diagnosed, Previously Untreated Glioblastoma.* Sponsor: Genetic Therapy, Inc./Novartis

Sole FDA Review Recommended by NIH/ORDA: 8-22-96

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**9608-158 (Open) Gene Therapy/Phase I-IB/Cancer/Melanoma or Sarcoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Plasmid DNA/Particle Mediated Gene Transfer (Accell®)/Cytokine/GM-CSF cDNA/Subcutaneous Injection**

Mahvi, David M.; University of Wisconsin Hospital and Clinics Comprehensive Cancer Center, Madison, Wisconsin; *Phase I/IB Study of Immunization with Autologous Tumor Cells Transfected with the GM-CSF Gene by Particle-Mediated Transfer in Patients with Melanoma or Sarcoma.*

Sole FDA Review Recommended by NIH/ORDA: 8-26-96

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**9605-159 (Open) Gene Marking/Cancer/Pediatric Malignancies/In Vitro/CD34+ Autologous Bone Marrow and Peripheral Blood/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant**

Heslop, Helen E.; Brenner, Malcolm K.; Krance, Robert A.; Baylor College of Medicine, Houston, Texas; *A Comparative Evaluation of the Utility of Hemopoietic Progenitor Cells Derived from Peripheral Blood vs Bone Marrow.*

Sole FDA Review Recommended by NIH/ORDA: 5-15-96

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**9609-160 (Closed) Gene Therapy/Phase I/Cancer/Prostate Adenocarcinoma/Immunotherapy/In Vivo/Vaccination/Vaccinia Virus/Prostate Specific Antigen/Intradermal Injection**

Kufe, Donald W.; and Eder, Joseph Paul; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Trial Of Recombinant Vaccinia Virus That Expresses PSA In Patients With Adenocarcinoma Of The Prostate.*

Sole FDA Review Recommended by NIH/ORDA: 9-18-96

Closed to subject entry: 1-31-00

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**9609-161 (Closed) Gene Therapy/Phase I/Cancer/Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Lipofectin(Gibco BRL)/B7-1(CD80) cDNA/Subcutaneous Injection**

Antonia, Scott J.; H. Lee Moffitt Cancer Center, Tampa, Florida; *Treatment of Small Cell Lung Cancer Patients In Partial Remission Or At Relapse With B7-1 Gene-Modified Autologous Tumor Cells As A Vaccine With Systemic Interferon Gamma.*

Sole FDA Review Recommended by NIH/ORDA: 10-10-96

Closed: 1-23-98. Protocol Never Initiated

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**9610-162 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Oncogene Regulation/HER-2/neu/ In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intratumoral Injection**

LaFollette, Suzanne; Rush/Presbyterian/St. Luke's Medical Center, Chicago, Illinois; Murray, James L.; M.D. Anderson Cancer Center, Houston, Texas; Yoo, George; Wayne State University, Detroit, Michigan; *A Phase I Multicenter Study of Intratumoral E1A Gene Therapy for Patients with Unresectable or Metastatic Solid Tumors that Overexpress HER-2/neu.* Sponsor: Targeted Genetics Corporation

Sole FDA Review Recommended by NIH/ORDA: 10-29-96

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**9610-163 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/MART-1 Melanoma Antigen/Intramuscular Injection**

Rosenberg, Steven A.; NIH, Bethesda, Maryland; *Phase I Trial In Patients With Metastatic Melanoma Of Immunization With A Recombinant Fowlpox Virus Encoding The MART-1 Melanoma Antigen.*

Sole FDA Review Recommended by NIH/ORDA: 5-23-96

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**9610-164 (Closed) Gene Therapy/Phase I/Cancer/Liver(Hepatic)Metastases/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection**

Sung, Max W.; and Woo, Savio L.C.; Mount Sinai Medical Center, New York, New York; *Phase I Trial of Adenoviral Vector Delivery of the Herpes Simplex Thymidine Kinase Gene by Intratumoral Injection Followed by Intravenous Ganciclovir in Patients with Hepatic Metastases.*

Sole FDA Review Recommended by NIH/ORDA: 11-12-96

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**9611-165 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/gp100 Melanoma Antigen/Intramuscular Injection**

Rosenberg, Steven A.; NIH, Bethesda, Maryland; *Phase I Trial In Patients With Metastatic Melanoma Of Immunization With A Recombinant Fowlpox Virus Encoding the GP100 Melanoma Antigen.*

NIH/ORDA Receipt Date: 11-13-96 . Sole FDA Review Recommended: 1-17-96

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**9611-166 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Vaccinia Virus/MART-1 Melanoma Antigen/Intramuscular Injection**

Rosenberg, Steven A.; NIH, Bethesda, Maryland; *Phase I Trial In Patients With Metastatic Melanoma Of Immunization With A Recombinant Vaccinia Virus Encoding the MART-1 Melanoma Antigen.*

NIH/ORDA Receipt Date: 11-13-96. Sole FDA Review Recommended: 1-17-96

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**9611-167 (Closed) Gene Therapy/Phase II/Cancer/Glioblastoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection**

Maria, Bernard, et al. (All #9608-157 sites are eligible to participate in this study.) *Prospective, Open-Label, Multicenter, Extension Trial for the Treatment of Recurrent Glioblastoma Multiforme with Surgery and Injection of Murine Cells Producing Herpes Simplex Thymidine Kinase Vector Followed by Intravenous Ganciclovir for Patients with Disease Progression Following Standard Treatment on Protocol GTI-0115.* Sponsor: Genetic Therapy, Inc./Novartis  
This protocol is an extension of #9608-157.

NIH/ORDA Receipt Date: 11-13-96. Sole FDA Review Recommended by NIH/ORDA: 1-6-97

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**9611-168 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL 1005/HLA-B7/Beta-2 Microglobulin cDNA/Direct Intratumoral Injection**

Hersh, Evan M.; Arizona Cancer Center, Tucson, Arizona; Klasa, Richard; British Columbia Cancer Agency, Vancouver, B.C., Canada; Gonzales, Rene; University of Colorado Cancer Center, Denver, Colorado; Silver, Gary; Northern California Melanoma Clinic, San Francisco, California; Thompson, John A.; U. of Washington Medical Center, Seattle, Washington; *Phase II Study of Immunotherapy of Metastatic Melanoma by Direct Gene Transfer*. Sponsor: Vical, Incorporated

NIH/ORDA Receipt Date: 11-26-96. Sole FDA Review Recommended by NIH/ORDA: 1-6-97

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**9611-169 (Closed) Gene Therapy/Phase I/II/Cancer/Solid Tumors/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL 1102/Cytokine/Interleukin-2 cDNA/Direct Intratumoral Injection**

Hersh, Evan, M.; Arizona Cancer Center, Tucson, Arizona; Rinehart, John; Scott and White Clinic, Temple, Texas; Rubin, Joseph; Mayo Clinic, Rochester, Minnesota; Sondak, Vernon K.; University of Michigan Medical Center, Ann Arbor, Michigan; Gonzales, Rene; University of Colorado Cancer Center, Denver, Colorado; Sobol, Robert E.; Sharp HealthCare, San Diego, California; and Forscher, Charles A.; Cedars-Sinai Comprehensive Cancer Center, Los Angeles, California; *Phase I/II Trial of Interleukin-2 DNA/DMRIE/DOPE Lipid Complex as an Immunotherapeutic Agent in Cancer by Direct Gene Transfer*. Sponsor: Vical, Incorporated

NIH/ORDA Receipt Date: 11-26-96. Sole FDA Review Recommended by NIH/ORDA: 1-17-97

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**9612-170 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Lung and Nasal Epithelial Cells/Cationic Liposome Complex/DOPE/CFTR cDNA/Aerosol Administration**

Sorscher, Eric; University of Alabama, Birmingham, Medical Center; *Safety and Efficiency of Gene Transfer of Aerosol Administration of a Single Dose of a Cationic Lipid/DNA Formulation to the Lungs and Nose of Patients with Cystic Fibrosis*. Sponsor: Genzyme Corporation

NIH/ORDA Receipt Date: 12-17-96. Sole FDA Review Recommended by NIH/ORDA: 1-6-97  
Completed: 12-97

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**9701-171 (Open) Non-Therapeutic/In Vivo/Intradermal Cells/Adenovirus/Serotype 5/E. coli Cytosine Deaminase/Intradermal Injection**

Harvey, Ben-Gary; and Crystal, Ronald G.; Rockefeller University Hospital, New York, New York; *Immune Response to Intradermal Administration of an Adenovirus Type 5 Gene Transfer Vector (Ad<sub>GV</sub>CD.10) in Normal Individuals*.

NIH/ORDA Receipt Date: 1-9-97. \*RAC Recommends Approval: 3-6-97/NIH Approval: 4-21-97

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**9701-172 (Closed) Gene Therapy/Phase I/Cancer/Testicular Cancer/Chemoprotection/In Vitro/G-CSF Mobilized Autologous CD34+ Peripheral Blood Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Bone Marrow Transplant**

Cornetta, Kenneth; and Abonour, Rafat; Indiana University Department of Medicine, Indianapolis, Indiana; *High Dose Carboplatin and Etoposide Followed by Transplantation with Peripheral Blood Stem Cells Transduced with the Multiple Drug Resistance Gene in the Treatment of Germ Cell Tumors - A Pilot Study*.

NIH/ORDA Receipt Date: 1-9-97. Sole FDA Review Recommended by NIH/ORDA: 2-26-97  
Closed to patient accrual: 3-15-00

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**9701-173 (Closed) Gene Therapy/Phase I/Cancer/Brain Tumors/Chemoprotection/In Vitro/Peripheral Blood CD34+ Cells/Retrovirus/O<sup>6</sup>-Methylguanine DNA Methyltransferase cDNA/Intravenous Infusion**

Croop, James; Indiana University School of Medicine, Indianapolis, Indiana; and Kieran, Mark, Dana-Farber Cancer Institute, Boston, Massachusetts; *A Pilot Study of Dose Intensified Procarbazine, CCNU, Vincristine(PCV) for Poor Prognosis Pediatric and Adult Brain Tumors Utilizing Fibronectin-Assisted, Retroviral-Mediated Modification of CD34+ Peripheral Blood Cells with O<sup>6</sup>-Methylguanine DNA Methyltransferase*.

NIH/ORDA Receipt Date: 1-13-97. Sole FDA Review Recommended by NIH/ORDA: 2-4-97  
Notification that trial is closed to new research participant enrollment: 2-20-01

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**9701-174 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Interleukin-2 cDNA/Neomycin Phosphotransferase cDNA/Immunoisolation Device/Subcutaneous Implantation**

Das Gupta, Tapas K.; University of Illinois at Chicago, Chicago, Illinois; *A Pilot Study Using Interleukin-2 Transfected Irradiated Allogeneic Melanoma Cells Encapsulated in an Immunoisolation Device In Patients with Metastatic Malignant Melanoma.*

NIH/ORDA Receipt Date: 1-13-97. Sole FDA Review Recommended by NIH/ORDA: 2-21-97

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**9701-175 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Stereotactic Injection**

Lieberman, Frank; Germano, Isabelle; and Woo, Savio; Mount Sinai Medical Center, New York, New York; *Gene Therapy for Recurrent Glioblastoma Multiforme: Phase I Trial of Intraparenchymal Adenoviral Vector Delivery of the HSV-TK Gene and Intravenous Administration of Ganciclovir.*

NIH/ORDA Receipt Date: 1-22-97. Sole FDA Review Recommended by NIH/ORDA: 2-12-97

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**9702-176 (Open) Gene Therapy/Phase I/III/Cancer/Prostate Adenocarcinoma/Immunotherapy/In Vivo/Vaccination/Vaccinia Virus/Prostate Specific Antigen/Intradermal Injection**

Sanda, Martin G.; University of Michigan Urology Clinics, Ann Arbor, Michigan; *A Phase I/II Clinical Trial Evaluating the Safety and Biological Activity of Recombinant Vaccinia-PSA Vaccine in Patients with Serological Recurrence of Prostate Cancer Following Radical Prostatectomy.*

NIH/ORDA Receipt Date: 2-19-97. Sole FDA Review Recommended by NIH/ORDA: 5-13-97

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**9702-177 (Open) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/Autologous Peripheral Blood Cells Mobilized by Cyclophosphamide and G-CSF/Retrovirus/Neomycin Phosphotransferase cDNA/Autologous Bone Marrow Transplant**

Verfaillie, Catherine; Mclvor, Scott; McCullough, Jeff; and McGlave, Philip; University of Minnesota, Minneapolis, Minnesota; *Autologous Marrow Transplantation for Chronic Myelogenous Leukemia Using Retrovirally Marked Peripheral Blood Progenitor Cells Obtained after In Vivo Cyclophosphamide/G-CSF Priming.*

NIH/ORDA Receipt Date: 2-21-97. Sole FDA Review Recommended by NIH/ORDA: 3-14-97

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**9703-178 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD34+ Autologous Cord Blood Cells/Retrovirus/Transdominant Trev/Intravenous**

Belmont, John W.; Texas Children's Hospital, Houston, Texas; *Phase I Clinical Trial of TREV Gene Therapy for Pediatric AIDS.*

NIH/ORDA Receipt Date: 3-10-97. Sole FDA Review Recommended by NIH/ORDA: 3-31-97

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**9703-179 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Carcinoembryonic Antigen/Intravenous**

Lyerly, Kim H.; Duke University Medical Center, Durham, North Carolina; *A Phase I Study of Active Immunotherapy With Carcinoembryonic Antigen RNA-Pulsed Autologous Human Cultured Dendritic Cells In Patients with Metastatic Malignancies Expressing Carcinoembryonic Antigen.*

NIH/ORDA Receipt Date: 3-14-97. Publicly Reviewed at the June 1997 RAC meeting.

Sole FDA Review Recommended by NIH/ORDA: 6-24-97

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**9703-180 (Open) Gene Therapy/Phase I/Other/Cubital Tunnel Syndrome/In Vivo/Autologous Muscle Cells/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Human Insulin-Like Growth Factor-1(hIGF-1)/Intramuscular Injection**

Netscher, David; Hand Clinic at the Veteran's Affairs (VA) Medical Center, Houston, Texas; *Phase I Single Dose-Ranging Study Of Formulated hIGF-1 Plasmid In Subjects With Cubital Tunnel Syndrome.* Sponsor: Gene Medicine, Inc.

NIH/ORDA Receipt Date: 3-17-97. Sole FDA Review Recommended: 4-7-97

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**9703-181 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD8 + and CD4+ T Lymphocytes/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous/Concurrent Interleukin-2 Therapy**

Connick, Elizabeth; University of Colorado Health Sciences Center, Denver, Colorado; Deeks, Steven G.; University of California, San Francisco General Hospital, San Francisco, California; Scadden, David; Massachusetts General Hospital (East), Charlestown, Massachusetts; Mitsuyasu, Ronald; University of California, Los Angeles Medical Center, Los Angeles, California; *A Phase II Study of the Activity and Safety of Autologous CD4-Zeta Gene-Modified T Cells With or Without Exogenous Interleukin-2 in HIV Infected Patients*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 3-19-97. Sole FDA Review Recommended: 4-18-97  
Notification from sponsor that trial is closed: 4-09-01

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**9703-182 (Open) Gene Therapy/Phase II/Monogenic Inherited Disorder/Cystic Fibrosis/Sinusitis/Correction/In Vivo/Maxillary Sinus Epithelial Cells/ Adeno-associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Maxillary Sinus Administration**

Gardner, Phyllis; Stanford University's General Clinical Research Center (GCRC), Palo Alto, California; *A Phase I/II Study of tgAAVCF for the Treatment of Chronic Sinusitis With Cystic Fibrosis*. Sponsor: Targeted Genetics Corporation

NIH/ORDA Receipt Date: 3-13-97. Sole FDA Review Recommended: 4-1-97

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**9703-183 (Closed) Gene Marking/Cancer/EBV-Positive Hodgkin Disease/In Vitro/EBV-Specific Hodgkin Disease/In Vitro/EBV-Specific Cytotoxic Lymphocytes/Retrovirus/Neomycin Phosphotransferase/Bone Marrow Transplant**

Straus, Stephan E.; National Institutes of Health, Bethesda, Maryland; *Administration of Neomycin Resistance Gene Marked EBV Specific Cytotoxic T-Lymphocytes To Patients With Relapsed EBV-Positive Hodgkin Disease*.  
Compassionate Case

NIH/ORDA Receipt Date: 3-19-97. Sole FDA Review Recommended by NIH/ORDA: 3-25-97  
Patient never treated (closed as of 11-18-97)

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**9703-184 (Closed) Gene Therapy/Phase I/Cancer/Prostate Cancer/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1102/Cytokine/Interleukin-2 cDNA/Intratumoral Injection**

Belldregun, Arie; University of California, Los Angeles, School of Medicine, Los Angeles, California; *A Phase I Study Evaluating the Safety and Efficacy of Interleukin-2 Gene Therapy Delivered by Lipid Mediated Gene Transfer (Leuvectin) in Prostate Cancer Patients*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 3-24-97. Sole FDA Review Recommended by NIH/ORDA: 5-21-97

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**9704-185 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Melanoma Cell/Canarypox Virus/Cytokine/Interleukin-12 cDNA/Intratumoral Injection**

Conry, Robert M.; University of Alabama at Birmingham, Birmingham, Alabama; *Phase Ib Trial of Intratumoral Injection of a Recombinant Canarypox Virus Encoding the Human Interleukin-12 Gene (ALVAC-hIL-12) in Patients with Surgically Incurable Melanoma*. Sponsor: NCI- Cancer Therapy Evaluation Program

NIH/ORDA Receipt Date: 4-1-97. Sole FDA Review Recommended by NIH/ORDA: 7-2-97  
Completed: 11-98

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**9704-186 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Cationic Liposome Complex/EDMPC/Intranasal Administration**

Noone, Peadar G.; Knowles, Michael R.; University of North Carolina at Chapel Hill, North Carolina; *A Double-Blind, Placebo Controlled, Dose Ranging Study to Evaluate the Safety and Biological Efficacy of the Lipid-DNA Complex GR213487B in the Nasal Epithelium of Adult Patients with Cystic Fibrosis*. Sponsor: Glaxo Wellcome Inc.

NIH/ORDA Receipt Date: 4-23-97. Sole FDA Review Recommended by NIH/ORDA: 5-13-97

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**9705-187 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection**

Hall, Simon J.; Woo, Savio L.C.; Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenoviral-Mediated Herpes Simplex Thymidine Kinase Gene Transduction in Conjunction with Ganciclovir Therapy as Neo-adjuvant Treatment for Patients with Clinically Localized (Stage T1c and T2b&c) Prostate Cancer Prior to Radical Prostatectomy.*

NIH/ORDA Receipt Date: 5-7-97. Sole FDA Review Recommended by NIH/ORDA: 5-28-97  
Closed to accrual: 11-12-01

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**9705-188 (Open) Gene Therapy/Phase I/Cancer/Chronic Myelogenous Leukemia/Chemoprotection/Tyr-22 Murine Dihydrofolate Reductase Gene/Antisense/Anti-b3a2BCR/ABL Gene/In Vitro/Autologous Peripheral Blood CD34+ Cells Mobilized by Cyclophosphamide and G-CSF/Retrovirus/Autologous Bone Marrow Transplant**

Verfaillie, Catherine; Mclvor, Scott; McCullough, Jeff; McGlave, Philip; University of Minnesota, Minneapolis, Minnesota; *Autologous Transplantation for Chronic Myelogenous Leukemia with Stem Cells Transduced with a Methotrexate Resistant DHFR and Anti-BCR/ABL Containing Vector and Post Transplant Methotrexate Administration.*

NIH/ORDA Receipt Date: 5-16-97. Sole FDA Review Recommended by NIH/ORDA: 6-6-97

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**9705-189 (Closed) Gene Therapy/Phase I/Cancer/Hepatocellular Carcinoma/Tumor Suppressor Gene/In Vivo/Autologous nTumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection**

Belani, Chandra P.; University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *Phase I Study of Percutaneous Injections of Adenovirus p53 Construct (Adeno-p53) for Hepatocellular Carcinoma.*

NIH/ORDA Receipt Date: 5-27-97. Sole FDA Review Recommended by NIH/ORDA: 9-19-97  
Closed: 3-7-00

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**9705-190 (Open) Gene Therapy/Phase I/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DOTMA-Cholesterol/Cytokine/Interleukin-2 cDNA/Intratumoral Injection**

O'Malley, Bert W.; Johns Hopkins Medical Institutions, Baltimore, Maryland; *A Double-Blind, Placebo-Controlled, Single Rising-Dose Study of the Safety and Tolerability of Formulated hIL-2 Plasmid in Patients with Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: Gene Medicine, Inc.

NIH/ORDA Receipt Date: 5-27-97. Sole FDA Review Recommended by NIH/ORDA: 6-16-97

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**9706-191 (Closed) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Direct Intratumoral Injection**

Gluckman, Jack L.; Gleich, Lyon L.; University of Cincinnati Medical Center, Cincinnati, Ohio; Swinehart, James M.; Colorado Medical Research Center, Denver, Colorado; Hanna, Ehab; University of Arkansas for Medical Sciences/Arkansas Cancer Research Center (UAMS), Little Rock, Arkansas; Castro, Dan J.; University of California, Los Angeles, Los Angeles, California; Gapany, Markus; Veterans Affairs Medical Center, Minneapolis, Minnesota; Carroll, William R.; University of Alabama at Birmingham, Birmingham, Alabama; Coltrera, Marc D.; University of Washington Medical Center, Seattle, Washington; Wolf, Gregory T.; University of Michigan Medical Center, Ann Arbor, Michigan; and Okuno, Scott; Mayo Clinic, Rochester, Minnesota; *Phase II Study of Immunotherapy by Direct Gene Transfer with Allovectin-7 for the Treatment of Recurrent or Metastatic Squamous Cell Carcinoma of the Head and Neck.* Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 6-6-97. Sole FDA Review Recommended by NIH/ORDA: 7-7-97

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**9706-192 (Open) Gene Therapy/Phase I/Cancer/Prostate/Tumor suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection**

Beldegrun, Arie; and Figlin, Robert.; UCLA School of Medicine, Los Angeles, California; *A Phase I Study in Patients with Locally Advanced or Recurrent Adenocarcinoma of the Prostate Using SCH58500 (rAd/p53) Administered by Intratumoral Injection.* Sponsor: Schering-Plough Corporation

NIH/ORDA Receipt Date: 6-9-97. Sole FDA Review Recommended by NIH/ORDA: 9-17-97

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**9706-193 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/CEA-Expressing Malignancies/In Vivo/Autologous Muscle Cells/Canarypox Virus/Vaccinia Virus/Carcinoembryonic Antigen cDNA/Intramuscular and Percutaneous Injection**

Marshall, John L.; Vincent T. Lombardi Cancer Research Center, Georgetown University Medical Center, Washington, D.C.; *A Pilot Study of Sequential Vaccinations with ALVAC-CEA and Vaccinia-CEA with the Addition of IL-2 and GM-CSF in Patients with CEA Expressing Tumors*. Sponsor: National Cancer Institute-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 6-18-97. Sole FDA Review Recommended by NIH/ORDA: 9-18-97

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**9706-194 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1 IIIB Envelope Protein/Intramuscular Injection**

Aboulafia, David; Virginia Mason Clinic, Seattle, Washington; Campbell, Thomas; University of Colorado Health Sciences Center, Denver, Colorado; Kumar, Princy; Georgetown University Medical Center, Washington, D.C.; Murphy, Robert; Northwestern University Medical School, Chicago, Illinois; Skolnik, Paul; New England Medical Center, Boston, Massachusetts; and Wheat, Joseph; Indiana University Hospital, Indianapolis, Indiana; *A Phase II, Randomized, Double Blind Placebo Controlled Study of Combination Drug Anti-Retroviral Therapy to Include a Reverse Transcriptase Inhibitor and a Protease Inhibitor Plus HIV-IT(V) or Placebo in HIV Patients with CD4+ Counts  $\geq 100$ , and HIV RNA  $\geq 1K$ , and  $\leq 10K$* . Sponsor: Chiron Corporation

NIH/ORDA Receipt Date: 6-23-97. Sole FDA Review Recommended by NIH/ORDA: 8-15-97  
5-10-00: IND no longer active

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**9706-195 (Closed) Gene Therapy/Phase I/Cancer/Immunotherapy/CEA-Expressing Malignancies/In Vivo/Vaccinia Virus/Carcinoembryonic Antigen cDNA/Intradermal and Subcutaneous Injections**

Conry, Robert M.; The University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Trial of a Recombinant Vaccinia-CEA (180 Kd) Vaccine Delivered by Intradermal Needle Injection Versus Subcutaneous Jet Injection in Patients with Metastatic CEA-Expressing Adenocarcinoma*. Sponsor: Drug Regulatory Affairs Branch, Cancer Therapy Evaluation Program (CTEP), Division of Cancer Treatment, Diagnosis and Centers, NCI, NIH

NIH/ORDA Receipt Date: 6-26-97. Sole FDA Review Recommended by NIH/ORDA: 9-5-97  
Completed: 7-98

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**9706-196 (Closed) Gene Therapy/Phase I/Monogenic Disease/Chronic Granulomatous Disease/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/gp91phox/Intravenous Infusion**

Croop, James; Indiana University School of Medicine, Indianapolis, Indiana; *Fibronectin-Assisted, Retroviral-Mediated Transduction of CD34+ Peripheral Blood Cells with gp91 phox in Patients with X-Linked Chronic Granulomatous Disease: A Phase I Study*.

NIH/ORDA Receipt Date: 6-30-97. Sole FDA Review Recommended by NIH/ORDA: 7-21-97  
Closed to enrollment: 4-06-04

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**9706-197 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Melanoma Cell/Canarypox Virus/B7(CD80)/Interleukin-12/Cytokine/Intratumoral Injection**

Conry, Robert M.; University of Alabama at Birmingham, Birmingham, Alabama; *Phase Ib Trial of Intratumoral Injection of a Recombinant Canarypox Virus Encoding Human B7.1 (ALVAC-hB7.1) or a Combination of ALVAC-hB7.1 and a Recombinant Canarypox Virus Encoding Human Interleukin-12 (ALVAC-hIL-12) in Patients with Surgically Incurable Melanoma*. Sponsor: National Cancer Institute-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 6-30-97. Sole FDA Review Recommended by NIH/ORDA: 9-5-97

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**9707-198 (Closed) Gene Therapy/Phase I/II/Cancer/Colorectal Carcinoma Expressing TAG-72/In Vitro/Autologous CD8+ and CD4+ T Lymphocytes/Retrovirus/CC49-Zeta T Cell Receptor/Intravenous Infusion**

Venook, Alan and Warren, Robert S.; University of California, San Francisco, California and Fisher, George; Stanford University, Palo Alto, California; *A Phase I/II Study of Autologous CC49-Zeta Gene-Modified T Cells and  $\alpha$ -Interferon in Patients with Advanced Colorectal Carcinomas Expressing the Tumor-Associated Antigen, TAG-72*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 7-7-97. Sole FDA Review Recommended by NIH/ORDA: 8-28-97  
Notification from sponsor that trial is closed: 4-09-01

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**9707-199 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Breast/Head and Neck Cancer/Cutaneous T-Cell Lymphoma/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-12/Intratumoral Injection**

Park, Chan H.; Samsung Medical Center, Seoul, Korea; Kim, Sunyoung; Seoul National University, Seoul, Korea; Lotze, Michael; Tahara, Hideaki; and Robbins, Paul; University of Pittsburgh, Pittsburgh, Pennsylvania; *IL-12 Gene Therapy Using Direct Injection of Tumors with Genetically Engineered Autologous Fibroblasts*.

NIH/ORDA Receipt Date: 7-22-97. Sole FDA Review Recommended by NIH/ORDA: 10-30-97

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**9707-200 (Open) Gene Therapy/Phase I/II/Cancer/Non-Hodgkin's B-Cell Lymphoma/Mantle Cell Lymphoma/Immunotherapy/In Vivo/Naked Plasmid DNA/Tumor Idiotypic/Intramuscular Injection**

Levy, Ronald; Stanford University School of Medicine, Stanford, California; *A Phase I/II Study of Vaccine Therapy for B-Cell Lymphoma Utilizing Plasmid DNA Coding for Tumor Idiotypic*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 7-24-97. Sole FDA Review Recommended by NIH/ORDA: 8-13-97

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**9707-201 (Open) Gene Therapy/Phase I/ Cancer/Ovarian/Immunotherapy/In Vitro/Autologous Tumor Cells/Canarypox Virus/B7.1 (CD80)/Intraperitoneal Injection**

Freedman, Ralph; The University of Texas, M.D. Anderson Cancer Center, Houston, Texas; *Intraperitoneal (IP) Autologous Therapeutic Tumor Vaccine (AUT-OV-ALVAC-hB7.1) plus IP rIFN- $\gamma$  for Patients with Ovarian Cancer. A Pilot Study*. Sponsor: NCI Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 7-28-97. Sole FDA Review Recommended by NIH/ORDA: 8-15-97

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**9707-202 (Closed) Gene Therapy/Phase I/Immunotherapy/Cancer/Melanoma/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Subcutaneous Injection**

Dranoff, Glenn and Soiffer, Robert; Dana-Farber Cancer Institute, Harvard Medical School, Boston, Massachusetts; *A Phase I Study of Vaccination with Autologous, Lethally Irradiated Melanoma Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor*.

NIH/ORDA Receipt Date: 7-28-97. Sole FDA Review Recommended by NIH/ORDA: 8-15-97  
Enrollment complete: 12-27-01

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**9707-203 (Closed) Gene Therapy/Phase I/Immunotherapy/Cancer/Non-Small Cell Lung Carcinoma (NSCLC)/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Subcutaneous Injection**

Dranoff, Glenn and Salgia, Ravi; Dana-Farber Cancer Institute, Harvard Medical School, Boston, Massachusetts; *A Phase I Study of Vaccination with Autologous, Lethally Irradiated Non-Small Cell Lung Carcinoma Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor*.

NIH/ORDA Receipt Date: 7-28-97. Sole FDA Review Recommended by NIH/ORDA: 8-15-97  
Enrollment complete: 12-27-01

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**9707-204 (Closed) Gene Therapy/Phase I/Monogenic Disease/Leukocyte Adherence Deficiency (LAD)/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/CD18/Intravenous Infusion**

Hickstein, Dennis and Bauer, Thomas R. National Institutes of Health, Bethesda, Maryland; *Retrovirus-Mediated Transfer of the cDNA for Human CD18 into Peripheral Blood Repopulating Cells of Patients with Leukocyte Adherence Deficiency*.

NIH/ORDA Receipt Date: 7-31-97. Sole FDA Review Recommended by NIH/ORDA: 9-17-97  
Closed: 9-17-00

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**9708-205 (Closed) Gene Therapy/Phase I/II/Cancer/Prostate/Immunotherapy/ In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection**

Simons, Jonathan W.; Johns Hopkins Oncology Center, Baltimore, Maryland; *Phase I/II Study of Allogeneic Human GM-CSF Gene Transduced Irradiated Prostate Cancer Cell Vaccines in Patients with Prostate Cancer*.

NIH/ORDA Receipt Date: 8-19-97. Sole FDA Review Recommended by NIH/ORDA: 9-9-97  
Closed: 7-23-01

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**9708-206 (Closed) Gene Therapy/Phase I/III/Cancer/Chronic Myelogenous Leukemia/Adoptive Immunotherapy/In Vitro/Donor CD8+ and CD4+ Lymphocytes/Retrovirus/Hygromycin Phosphotransferase-Herpes Simplex Thymidine Kinase Fusion Gene/Intravenous Infusion**

Flowers, Mary E. D. and Riddell, Stanley; Fred Hutchinson Cancer Research Center, Seattle, Washington; *Infusion of Polyclonal HyTK (hygromycin phosphotransferase and HSV thymidine kinase gene)-transduced Donor T Cells for Adoptive Immunotherapy in Patients with Relapsed CML after Allogeneic Stem Cell Transplant: Phase I-II Clinical Trial.*

NIH/ORDA Receipt Date: 8-19-97. Sole FDA Review Recommended by NIH/ORDA: 9-26-97  
Closed to new accrual: 4-24-00.

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**9708-207 (Closed) Gene Therapy/Phase I/Cancer/Colorectal/Immunotherapy/In Vivo/Autologous Tumor Cells/Canarypox Virus/Carcinoembryonic Antigen/B7.1 (CD80)/Intradermal Scarification**

Kaufman, Howard L.; Albert Einstein Cancer Center, Bronx, New York; *Phase I Clinical Trial of a Recombinant ALVAC-CEA-B7 Vaccine in the Treatment of Advanced Colorectal Carcinoma.* Sponsor: National Cancer Institute-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 8-21-97. Sole FDA Review Recommended by NIH/ORDA: 11-25-97  
Closed: 2-99.

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**9708-208 (Open) Gene Therapy/Phase I/Cancer/Mesothelioma/Pro-Drug/In Vivo/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Herpes Simplex Virus Thymidine Kinase/Ganciclovir/Intrapleural Administration**

Schwarzenberger, Paul; Louisiana State University Medical Center, New Orleans, Louisiana; *The Treatment of Malignant Pleural Mesothelioma with a Gene-Modified Cancer Vaccine: A Phase I Study.*

NIH/ORDA Receipt Date: 8-25-97. Sole FDA Review Recommended by NIH/ORDA: 9-16-97

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**9708-209 (Closed) Non-Therapeutic/In Vivo/Bronchial Epithelial Cells/Adenovirus/Serotype 5/E. coli Cytosine Deaminase/Intrabronchial Administration**

Harvey, Ben-Gary and Crystal, Ronald G.; Rockefeller University Hospital, New York, New York; *Systemic and Respiratory Immune Response to Administration of an Adenovirus Type 5 Gene Transfer Vector (Ad<sub>GV</sub>CD.10).*

NIH/ORDA Receipt Date: 8-26-97. Publicly Reviewed at the December 16, 1997 RAC meeting  
Closed: 09-19-00

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**9709-210 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1005/HLA-B7/β2-Macroglobulin cDNA/Direct Intratumoral Injection**

Gonzales, Rene; University of Colorado Cancer Center, Denver, Colorado; Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; Deisseroth, Albert, Yale University, New Haven, Connecticut; Paciucci, Paolo A., Mt. Sinai Medical Center, New York, New York; Hutchins, Laura F., University of Arkansas for Medical Sciences, Little Rock, Arkansas; Galanis, Evan, Mayo Clinic, Rochester, Minnesota; Schaefer, Paul L., Toledo Clinic, Toledo, Ohio; Amatruda, Thomas, Virginia Piper Cancer Institute Abbott Northwestern Hospital, Minneapolis, Minnesota; Kuzel, Timothy, Northwestern Medical Faculty Foundation Northwestern Memorial Hospital, Chicago, Illinois; Blum, Ronald H., Beth Israel Medical Center, Phillips Ambulatory Care Center, New York, New York; Whitman, Eric D., The Melanoma Center of St. Louis, Saint Louis, Missouri; Cobb, Patrick, Billings Interhospital Oncology Project, Billings, Montana; Amin, Bipinkumar, Mid Dakota Clinic, Bismarck, North Dakota; Chowhan, Naveed, Cancer Care Center Incorporated, New Albany, Indiana; Lutzky, Jose, Mount Sinai Medical Center, Miami, Florida; Amatruda, Thomas, North Memorial Healthcare, Hubert H. Humphrey Cancer Center, Robbinsdale, Minnesota; Patel, Ravi, Comprehensive Blood and Cancer Center, Bakersfield, California; Dobbs, Tracy W., Baptist Hospital of East Tennessee, Knoxville, Tennessee; Ahmed, Fakhiuddin, HemOnCare, P.C., Brooklyn, New York; Thant, Myo, Maryland Hematology/Oncology Associates, Baltimore, Maryland; Stark, James J., Maryview Medical Center, Portsmouth, Virginia; Arena, Francis, Arena Oncology Associates, Great Neck, New York; Soori, Gamini, Alegant Health, Bergan Mercy Medical Center, Omaha, Nebraska; Samlowski, Wolfram, University of Utah Health Sciences Center; Huntsman Cancer Institute, Salt Lake City, Utah; Polikoff, Jonathan A., Kaiser Permanente Medical Group, San Diego, California; Hawkins, Michael, Washington Hospital Center, Washington Cancer Institute, Washington, D.C.; Richart, John, Saint Louis University Health Sciences Center, St. Louis, Missouri; Patel, Taral, Columbus, Community Clinical Oncology Program, Columbus, Ohio; Levine, Edward, Wake Forest University School of Medicine, Winston Salem, North Carolina; Richards, Jon, Lutheran General Hospital, Park Ridge, Illinois; Thompson, John A., University of Washington Medical Center, Seattle, Washington; and Schwarzenberger, Paul, Louisiana State University Health Sciences Center, New Orleans, Louisiana; *Compassionate Use Protocol for Retreatment with Allovectin-7 Immunotherapy for Metastatic Cancer by Direct Gene Transfer.* Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 9-8-97. Sole FDA Review Recommended by NIH/ORDA: 9-26-97

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**9708-211 (Closed) Gene Therapy/Phase I/Monogenetic Disease/Canavan Disease/In Vivo/Autologous Brain Cells/Plasmid DNA/Adeno-associated Virus/Poly-L-Lysine/Cationic Liposome Complex/DC-Chol/DOPE/Aspartoacylase cDNA/Intracranial (Ommaya Reservoir) Administration**

Seashore, Margretta R.; Yale University, New Haven, Connecticut; *Gene Therapy of Canavan Disease: Retreatment of Previously Treated Children.*

NIH/ORDA Receipt Date: 8-28-97. Publicly Reviewed at the December 16, 1997 RAC meeting  
Closed: 5-22-00

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**9709-212 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Vical-1102/Interleukin-2 cDNA/Intratatumoral Injection**

Gonzalez, Rene; University of Colorado Health Sciences Center, Denver, Colorado; Hersh, Evan M.; Arizona Cancer Center, Tucson, Arizona; Rubin, Joseph; Mayo Clinic, Rochester, Minnesota; and Thompson, John A.; University of Washington Medical Center, Seattle, Washington; *Phase I Study of Direct Gene Transfer of HLA-B7 Plasmid DNA/DMRIE/DOPE Lipid Complex (Allovectin-7) with IL-2 Plasmid DNA/DMRIE/DOPE Lipid Complex (Leuvectin) as an Immunotherapeutic Regimen in Patients with Metastatic Melanoma.* Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 9-18-97. Sole FDA Review Recommended by NIH/ORDA: 10-8-97

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**9709-213 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD8+ T Cells/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous**

Deeks, Steven G.; University of California, San Francisco General Hospital, San Francisco, California; *A Phase II Study of Autologous CD4-Zeta Gene-Modified T Cells in HIV-Infected Patients with Undetectable Plasma Viremia on Combination Antiretroviral Drug Therapy.* Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 9-22-97. Sole FDA Review Recommended by NIH/ORDA: 10-10-97

Study closed to new accrual, follow-up is continuing: 7-13-01

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**9709-214 (Open) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53**

Breau, Randall L.; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Clayman, Gary L.; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; Yoo, George H.; Wayne State University/Barbara Ann Karmanos Cancer Institute, Detroit, Michigan; Medina, Jesus E.; University of Oklahoma Health Sciences Center, Oklahoma City, Oklahoma; Murphy, Barbara S.; Vanderbilt University Medical Center, Nashville, Tennessee; Goodwin, W. Jarrard; University of Miami Hospitals and Clinics, Miami, Florida; Weber, Jeffery S.; University of Southern California, Los Angeles, California; Schuller, David E.; Ohio State University Medical Center, Columbus, Ohio; Bukowski, Ronald M.; The Cleveland Clinic Foundation, Cleveland, Ohio; Hamm, John; University of Louisville Health Sciences Center, Louisville, Kentucky; Agarwala, Sanjiv; University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; Hochster, Howard S.; New York University Medical Center, New York, New York; Dietz, Andreas; University of Heidelberg, Heidelberg, Germany; Eßer, Dirk; Ear, Nose and Throat Clinic, Erfurt, Germany; and Flood, William A.; Milton S. Hershey Medical Center, Hershey, Pennsylvania; *A Phase II Multi-Center, Open Label, Randomized Study to Evaluate Effectiveness and Safety of Two Treatment Regimens of Ad5CMV-p53 Administered by Intra-Tumoral Injections in 78 Patients with Recurrent Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: Aventis (formerly Gencell)

NIH/ORDA Receipt Date: 9-22-97. Sole FDA Review Recommended by NIH/ORDA: 10-21-97

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**9709-215 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Autologous Tumor Cells/Canarypox Virus/Carcinoembryonic Antigen/B7.1 (CD80)/Intramuscular and Intradermal Injections**

von Mehren, Margaret; Fox Chase Cancer Center, Philadelphia, Pennsylvania; *Phase I/Pilot Study of ALVAC-CEA-B7.1 Immunization in Patients with Advanced Adenocarcinoma Expressing CEA.* Sponsor: National Cancer Institute - Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 9-24-97. Sole FDA Review Recommended by NIH/ORDA: 10-28-97

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**9709-216 (Open) Gene Therapy/Phase I/Cancer/Breast/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Cutaneous or Subcutaneous**

von Mehren, Margaret; Fox Chase Cancer Center, Philadelphia, Pennsylvania; *Phase I/Pilot Study of p53 Intralesional Gene Therapy with Chemotherapy in Breast Cancer.* Sponsor: National Cancer Institute - Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 9-24-97. Sole FDA Review Recommended by NIH/ORDA: 10-28-97

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**9710-217 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection**

Logothetis, Christopher J.; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Tolerance and Efficacy Study of Intraprostatic INGN 201 Followed by Pathological Staging and Possible Radical Prostatectomy in Patients with Locally Advanced Prostate Cancer*. Sponsor: Introgen Therapeutics, Inc.

NIH/ORDA Receipt Date: 10-3-97. Sole FDA Review Recommended by NIH/ORDA: 11-6-97

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**9710-218 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Hammerhead Ribozyme/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Tat and Rev Hammerhead Ribozyme/Intravenous**

Krishnan, Amrita and Zaia, John, A.; City of Hope Medical Center, Duarte, California; *High Dose Chemotherapy and Autologous Peripheral Stem Cell Transplantation for HIV Lymphomas: A Phase IIa Study of Comparative Marking Using a Ribozyme Gene and a Neutral Gene*. Sponsor: Ribozyme Pharmaceuticals, Inc.

NIH/ORDA Receipt Date: 10-6-97. Sole FDA Review Recommended by NIH/ORDA: 10-27-97  
Closed to new enrollment: 12-17-99

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**9710-219 (Open) Gene Therapy/Phase I/Cancer/Bladder/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intravesical Administration**

Pagliaro, Lance C.; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Trial of Intravesical Ad-p53 Treatment in Locally Advanced and Metastatic Bladder Cancer*.

NIH/ORDA Receipt Date: 10-21-97. Sole FDA Review Recommended by NIH/ORDA: 11-10-97

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**9710-220 (Open) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Bronchoscopy or Percutaneous Intratumoral Injection**

Dobbs, Tracy W.; East Tennessee Oncology/Hematology, P.C., Knoxville, Tennessee; *A Phase II Gene Therapy Study in Patients with Non-Small Cell Lung Cancer Using SCH 58500 (rAd/p53) in Combination with Chemotherapy for Multiple Cycles*. Sponsor: Schering Plough Research Institute

NIH/ORDA Receipt Date: 10-31-97. Not Selected for RAC Public Review: 12-15-97

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**9711-221 (Closed) Gene Therapy/Phase I/Other/ Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Administration**

Crystal, Ronald G.; The New York Hospital-Cornell Medical Center, New York, New York; *Phase I Study of Direct Administration of a Replication-Deficient Adenovirus Vector (Ad<sub>GV</sub>/VEGF121.10) Containing the VEGF121 cDNA to the Ischemic Myocardium of Individuals with Life Threatening Diffuse Coronary Artery Disease*. Sponsor: Parke-Davis Pharmaceutical Research.

NIH/ORDA Receipt Date: 11-4-97. Publicly Reviewed at the December 16, 1997 RAC meeting  
Completed, long-term follow-up continued: October 1999

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**9711-222 (Closed) Gene Therapy/Phase I/Monogenetic Disease/Canavan Disease/In Vivo/Autologous Brain Cells/Plasmid DNA/Adeno-Associated Virus/Protamine/Cationic Liposome Complex/DC-Cholesterol-DOPE/Aspartoacylase cDNA/Intracranial (Ommaya Reservoir)**

Freese, Andrew; Thomas Jefferson University, Philadelphia, Pennsylvania; *Gene Therapy of Canavan Disease*.

NIH/ORDA Receipt Date: 11-12-97. Not Selected for RAC Public Review: 1-26-98  
Closed: 5-22-00

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**9712-223 (Open) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Allogeneic Neuroblastoma Cell Lines/Retrovirus/Cytokine/Interleukin-2 (IL-2)/Plasmid/Electroporation/Chemokine/Lymphotactin/Subcutaneous Injection**

Hale, Gregory; St. Jude Children's Research Hospital, Memphis, Tennessee; *Phase I Study of Chemokine and Cytokine Gene Modified Allogeneic Neuroblastoma Cells for Treatment of Relapsed/Refractory Neuroblastoma Using a Retroviral Vector*.

NIH/ORDA Receipt Date: 12-3-97. Not Selected for RAC Public Review: 12-29-97

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**9712-224 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous Tumor Cells (Non-Irradiated)/Type 5 Adenovirus/Cytokine/Interleukin-2 (IL-2)/Chemokine/Lymphotactin/Subcutaneous Injection**

Hale, Gregory; St. Jude Children's Research Hospital, Memphis, Tennessee; *Phase I Study of Chemokine and Cytokine Gene Modified Autologous Neuroblastoma Cells for Treatment of Relapsed/Refractory Neuroblastoma Using an Adenoviral Vector.*

NIH/ORDA Receipt Date: 12-3-97. Not Selected for RAC Public Review: 12-29-97  
Closed to new enrollment: 3-17-04

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**9712-225 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/Antisense TAR/Transdominant Rev/Intravenous**

Isola, Luis M.; Mount Sinai Medical Center, New York, New York; *A Phase I Trial of Autologous and Allogeneic Bone Marrow Transplantation with Genetically Marked Cells for the Treatment of HIV Associated Lymphoid Malignancies.*

NIH/ORDA Receipt Date: 12-15-97. Not Selected for RAC Public Review: 1-7-98  
IND withdrawn: 4-4-00

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**9712-226 (Open) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injections**

Dreicer, Robert; University of Iowa College of Medicine, Iowa City, Iowa; Simon, George R.; University of Colorado Health Sciences Center, Denver, Colorado; Williamson, Stephen; University of Kansas Medical Center, Kansas City, Kansas; VanECHO, David A.; University of Maryland School of Medicine, Baltimore, Maryland; Rosen, Fred; University of Illinois at Chicago Hospitals & Clinics; Endicott, James N.; University of South Florida, Tampa, Florida; Bier-Laning, Carol M.; University of Texas Southwestern Medical Center at Dallas, Dallas, Texas; Minn, Heikki; Turku University Central Hospital, Turku Finland; Guertin, Louis; CHUM - Pavilion Notre-Dame, Montreal, Quebec; Liu, Fei-Fei; Princess Margaret Hospital, Toronto, Ontario; Wadler, Scott; Montefiore Medical Center, Albert Einstein College of Medicine; Bronx, New York; Goss, Glenwood D.; Ottawa Regional Cancer Centre, Ottawa, Ontario; Saarilanti, Kauko; Helsinki University Central Hospital, Helsinki Finland; Mudad, Raja; Tulane University Medical Center, New Orleans, Louisiana; Spiro, Jeffrey; University of Connecticut Health Center, Farmington, Connecticut; Zielinski, Christoph; University of Vienna; Link, Brian; University of Iowa Hospital and Clinics, Iowa City, Iowa; and Truelson, John; University of Texas Southwestern Medical School, Dallas, Texas; *A Phase II, Multi-Center, Open Label, Study to Evaluate Effectiveness and Safety of Ad5CMV-p53 Administered by Intra-Tumoral Injections in 39 Patients with Recurrent Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: Aventis (formerly Gencell)

NIH/ORDA Receipt Date: 12-17-97. Not Selected for RAC Public Review: 1-9-98

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**9801-227 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Head and Neck Cancer/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-12 cDNA/Neomycin Phosphotransferase cDNA/Intratumoral Injection**

Lotze, Michael T.; University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; *IL-12 Gene Therapy Using Direct Injection of Tumors with Genetically Engineered Autologous Fibroblasts (A Phase II Study).*

NIH/ORDA Receipt Date: 1-2-98. Not Selected for RAC Public Review: 2-18-98  
Protocol is terminated: 11-1-01

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**9801-228 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/ Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Acyclovir/Intraperitoneal Injection**

Kieback, Dirk G.; Baylor College of Medicine, Houston, Texas; *Phase I Study of Concomitant Adenovirus-Mediated Transduction of Ovarian Cancer with HSV-tk Gene Followed by Intravenous Administration of Acyclovir and Chemotherapy with Topotecan in Patients after Optimal Debulking Surgery for Recurrent Ovarian Cancer.*

NIH/ORDA Receipt Date: 1-14-98. Not Selected for RAC Public Review: 2-5-98

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**9801-229 (Open) Gene Therapy/Phase I/Cancer/Prostate/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection**

Kadmon, Dov; Baylor College of Medicine, Houston, Texas; *Neoadjuvant Pre-radical Prostatectomy Gene Therapy (HSV-tk Gene Transduction Followed by Ganciclovir) in Patients with Poor Prognostic Indicators.*

NIH/ORDA Receipt Date: 1-16-98. Not Selected for RAC Public Review: 2-13-98

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**9801-230 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/Antisense TAR/Antisense tat | rev/In Vitro/CD34+ Cells/Intravenous**

Cowan, Morton J. and Conant, Marcus A.; University of California, San Francisco, San Francisco, California; *Evaluation of the Safety and Effects of Ex Vivo Modification and Re-infusion of CD34+ Cells by an Antisense Construct Against HIV-1 in a Retroviral Vector*. Sponsor: Enzo Therapeutics, Inc.

NIH/ORDA Receipt Date: 1-20-98. Not Selected for RAC Public Review: 3-26-98  
Closed to enrollment, follow-up is continuing: 06-24-03

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**9802-231 (Closed) Gene Therapy/Phase I/II/Monogenic Disease/Chronic Granulomatous Disease/In Vitro/CD 34+ Autologous Peripheral Blood Cells/Retrovirus/p47phox/gp91phox/Intravenous**

Malech, Harry L.; National Institutes of Health, Bethesda, Maryland; *Gene Therapy Approach for Chronic Granulomatous Disease*.

NIH/ORDA Receipt Date: 2-2-98. Not Selected for RAC Public Review: 2-20-98  
Closed to enrollment: 4-12-04

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**9802-232 (Closed) Gene Therapy/Phase I/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Administration**

Isner, Jeffrey M.; Tufts University School of Medicine, Boston, Massachusetts; *Gene Therapy for Myocardial Angiogenesis*.

NIH/ORDA Receipt Date: 2-3-98. Publicly Reviewed at the June 18, 1998 RAC meeting  
Follow-up has been completed: 11-29-01

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**9802-233 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection**

Dreicer, Robert; the University of Iowa Hospitals and Clinics, Iowa City, Iowa; Seigler, Hilliard; Duke University Medical Center, Durham, North Carolina; Rubin, Joseph; Mayo Clinic, Rochester, Minnesota; DeConti, Robert; H. Lee Moffitt Cancer Center, Tampa, Florida; Gonzalez, Rene; the University of Colorado Cancer Center, Denver Colorado; Macdonald, John S.; Saint Vincent's Hospital and Medical Center, New York, NY; Hutchins, Laura; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Samlowski, Wolfram E.; the University of Utah Health Sciences Center, Salt Lake City, Utah; Bearden, James D.; Spartanburg Regional Medical Center, Spartanburg, South Carolina; Atkins, Michael B.; Beth Israel Medical Center, Boston, Massachusetts; Schwarzenberger, Paul O.; Louisiana State University Medical Center, New Orleans, Louisiana; Deisseroth, Albert; Yale University School of Medicine, New Haven, Connecticut; Blum, Ronald H.; Beth Israel Medical, New York, New York; Lutzky, Jose; Mount Sinai Medical Center, Miami, Florida; and Wallach, Sabina R.; Scripps Memorial Hospital, San Diego, La Jolla, and Encinitas, California; *Phase II Study of Direct Gene Transfer of HLA-B7 Plasmid DNA/DMRIE/DOPE Lipid Complex (Allovectin-7) as an Immunotherapeutic Agent in Patients with Stage III or IV Melanoma with No Treatment Alternatives*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 2-9-98. Not Selected for RAC Public Review: 8-28-98

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**9802-234 (Closed) Gene Therapy/Phase III/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection**

Thompson, John A.; University of Washington, Seattle, Washington; Dreicer, Robert; the University of Iowa Hospitals and Clinics, Iowa City, Iowa; Seigler, Hilliard; Duke University Medical Center, Durham, North Carolina; Galanis, Evanthea; Mayo Clinic, Rochester, Minnesota; DeConti, Robert; H. Lee Moffitt Cancer Center, Tampa, Florida; Macdonald John S.; Saint Vincent's Hospital and Medical Center, New York, NY; Hutchins, Laura; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Samlowski, Wolfram E.; the University of Utah Health Sciences Center, Salt Lake City, Utah; Bearden, James D.; Spartanburg Regional Medical Center, Spartanburg, South Carolina; Atkins, Michael B.; Beth Israel Medical Center, Boston, Massachusetts; Gibbs, John and Aleksowicz, Leslie; Roswell Park Cancer Institute, Buffalo, New York; Schwarzenberger, Paul O.; Louisiana State University Medical Center, New Orleans, Louisiana; Ernstoff, Marc, Dartmouth Hitchcock Medical Center, Lebanon, New Hampshire; Campbell, Laura, Louisiana State University Medical Center, Shreveport, Louisiana; Levine, Edward; Wake Forest University Medical Center, Winston-Salem, North Carolina; Schuchter, Lynn M.; University of Pennsylvania Cancer Center, Philadelphia, Pennsylvania; Deisseroth, Albert; Yale University School of Medicine, New Haven, Connecticut; Paciucci, Paolo A.; Mount Sinai Medical Center, New York, New York; Richart, John; Saint Louis University Health Sciences Center, St. Louis, Missouri; Meyskens Jr., Frank L.; University of California, Irvine, Orange, California; Blum, Ronald H.; Beth Israel Medical, New York, New York; Amatruda, Thomas; Virginia Piper Cancer Institute Abbott Northwestern Hospital, Minneapolis, Minnesota; Kuzel, Timothy; Northwestern Medical Faculty Foundation and Northwestern Memorial Hospital, Chicago, Illinois; Hawkins, Michael; Washington Cancer Institute, Washington, DC; Whitman, Eric D.; The Melanoma Center of St. Louis, Saint Louis, Missouri; Cobb, Patrick; Billings Interhospital Oncology Project, Billings, Montana; Amin, Bipinkumar; Mid Dakota Clinic, Bismarck, North Dakota; Chowhan, Naveed; Cancer Care Center Incorporated, New Albany, Indiana; Lutzky, Jose; Mount Sinai Medical Center, Miami, Florida; Amatruda, Thomas; North Memorial Healthcare, Hubert H. Humphrey Cancer Center, Robbinsdale, Minnesota; Patel, Ravi; Comprehensive Blood and Cancer Center, Bakersfield, California; Dobbs, Tracy W.; Baptist Hospital of East Tennessee, Knoxville, Tennessee; Ahmed, Fakhuddin; HemOnCare, P.C., Brooklyn, New York; Thant, Myo; Maryland Hematology/Oncology Associates, Baltimore, Maryland; Stark, James J.; Maryview Medical Center, Portsmouth, Virginia; Arena, Francis; Arena Oncology Associates, Great Neck, New York; Brotherton, Timothy; Danville Hematology and Oncology, Inc. Danville Diagnostic Imaging Center, Danville, Virginia; Brouillard, Robert P.; Scripps Memorial Hospital, La Jolla, Encinitas, and El Cajon, California; Polikoff, Jonathan A.; Kaiser Permanente Medical Group, San Diego, California; Ritch, Paul S.; Medical College of Wisconsin and Froedtert Memorial Lutheran Hospital, Milwaukee, Wisconsin; Bernstein, Joel I.; Scripps Memorial Hospital, La Jolla, Encinitas, El Cajon, California; Richards, Jon; Lutheran General Hospital, Park Ridge, Illinois; and Giguere, Jeffrey; Hematology and Oncology Associates, Greenville, South Carolina; *A Controlled, Randomized Phase III Trial Comparing the Response to Dacarbazine with and without Allovectin-7 in Patients with Metastatic Melanoma*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 2-9-98. Not Selected for RAC Public Review: 7-20-98

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**9802-235 (Open) Gene Therapy/Phase I/Cancer/Brain Tumors/Glioblastoma/Vector-Directed Cell Lysis/In Vivo/Autologous Tumor Cells/Herpes Simplex Virus Type I/Tumor Lysis/Intratumoral Injection**

Markert, James; University of Alabama, Birmingham, Alabama; and Medlock, Michael; Georgetown University Medical Center, Washington, D.C.; *A Dose Escalating Phase I Study of the Treatment of Malignant Glioma with G207, a Genetically Engineered HSV-1*. Sponsor: NeuroVir, Inc.

NIH/ORDA Receipt Date: 2-10-98. Publicly Reviewed at the June 18, 1998 RAC meeting

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**9802-236 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Autologous Tumor Cells/Adenovirus Type 5/Replication-competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intratumoral Injection**

Simons, Jonathan W.; Johns Hopkins University School of Medicine, Baltimore, Maryland; *A Phase I Study of the Intraprostatic Injections of CN706, a Prostate-Specific Antigen Gene-Regulated Cytolytic Adenovirus, in Patients with Locally Recurrent Cancer Following Definitive Radiotherapy*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 2-13-98. Publicly Reviewed at the June 19, 1998 RAC meeting

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**9802-237 (Closed) Gene Therapy/Phase I/Rheumatoid Arthritis/In Vivo/Autologous Synovial Cells/Naked Plasmid DNA/Herpes Simplex Virus Thymidine Kinase Gene/Ganciclovir/Intra-Articular Administration**

Roessler, Blake J; The University of Michigan Medical Center, Ann Arbor, Michigan; *Molecular Synovectomy by In Vivo Gene Transfer: A Phase I Trial*.

NIH/ORDA Receipt Date: 2-13-98. Publicly Reviewed at the June 18, 1998 RAC meeting  
Closed to new enrollment: 5-15-02; follow-up is continuing

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**9802-238 (Closed) Gene Therapy/Phase I-II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration**

Lee, Joon S.; University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *Phase 1/2 Study of the Effects of Ascending Doses of Adenovirus Mediated Human FGF-4 Gene Transfer in Patients with Stable Exertional Angina*. Sponsor: Berlex Laboratories, Inc.

NIH/ORDA Receipt Date: 2-24-98. Publicly Reviewed at the June 18, 1998 RAC meeting  
Completed: 06-03

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**9802-239 (Closed) Gene Therapy/Phase I-II/Cancer/Hepatic Metastasis of Colorectal Carcinoma/Immunotherapy/In Vitro/Autologous CD4+ and CD8+ Lymphocytes/Retrovirus/CC49-Zeta T Cell Receptor/Hepatic Artery Infusion**

Bergsland, Emily K.; University of California, San Francisco, San Francisco, California; *A Phase I/II Study of Hepatic Infusion of Autologous CC49-Zeta Gene-Modified T Cells in Patients with Hepatic Metastasis from Colorectal Cancer*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 2-25-98. Not Selected for RAC Public Review: 3-17-98

Notification from sponsor that trial is closed: 4-09-01

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**9803-240 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection**

Rom, William N.; New York University School of Medicine, New York, New York; and Woo, Savio L.C.; Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenoviral Vector Delivery of the Herpes Simplex Thymidine Kinase Gene by Intratumoral Injection Followed by Intravenous Ganciclovir in Patients with Advanced Non-Small Cell Lung Cancer*.

NIH/ORDA Receipt Date: 3-3-98. Not Selected for RAC Public Review: 3-23-98

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**9803-241 (Closed) Gene Therapy/Phase I-II/Cancer/Chronic Myelogenous Leukemia/Multiple Myeloma/Non-Hodgkin's Lymphoma/Chronic Lymphocytic Leukemia/Adoptive Immunotherapy/In Vitro/Sibling Peripheral Blood Lymphocytes/Retrovirus/Herpes Simplex Virus Thymidine Kinase/Ganciclovir/Intravenous Infusion**

Bensinger, William I.; University of Washington School of Medicine, Seattle, Washington; Parker, Pablo M.; City of Hope National Medical Center, Duarte, California; Henslee-Downey, Peggy J. and Abhyankar, Sunil; Richland Memorial Hospital, University of South Carolina, Columbia, South Carolina; Giralto, Sergio; University of Texas, M.D. Anderson Cancer Center, Houston, Texas; Cornetta, Kenneth; Indiana University-Purdue University, Indianapolis, Indiana; and Carabasi, Matthew; The University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I/II Outpatient, Multicenter, Intrapatient, Multiple Dose Escalation Study of Herpes Simplex Virus Thymidine Kinase (HSV-TK) Transduced Mononuclear Cells in Subjects with Persistent or Relapsed Chronic Myelogenous Leukemia, Chronic Lymphocytic Leukemia, Multiple Myeloma, and Non-Hodgkin's Lymphoma after HLA-Matched Sibling Allogeneic Stem Cell Transplant*. Sponsor: Chiron Corporation

NIH/ORDA Receipt Date: 3-27-98. Not Selected for RAC Public Review: 4-17-98

5-5-00: IND no longer active

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**9803-242 (Closed) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous Leukemic Cells/Adenovirus/Serotype 5/CD 154 cDNA/Intravenous Infusion**

Kipps, Thomas J.; University of California, San Diego, San Diego, California; *A Phase I Study of CD 154 Gene-Transduced Leukemia Cells in Patients with Chronic Lymphocytic Leukemia*.

NIH/ORDA Receipt Date: 3-30-98. Not Selected for RAC Public Review: 4-17-98

Notification from Immunogenex, now the sponsor of this trial, that study has been completed: 3-20-01

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**9804-243 (Closed) Gene Therapy/Phase I/Other/Peripheral Arterial Disease/In Vivo/Ischemic Lower Limb/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection**

Crystal, Ronald G.; Cornell University Medical College, New York, New York; Deitcher, Steven and Goldman, Corey; The Cleveland Clinic Foundation, Cleveland, Ohio; Rajagopalan, Sanjay; The University of Michigan, Ann Arbor, Michigan; Mohler III, Emile R.; University of Pennsylvania Health System, Philadelphia, Pennsylvania; and Trachtenberg, Jeffrey; University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *Phase I Study of Direct Administration of a Replication Deficient Adenovirus vector (Ad<sub>GV</sub>VEGF121.10) Containing the VEGF121 cDNA to the Ischemic Lower Limb of Individuals with Peripheral Vascular Disease*. Sponsor: Parke-Davis Pharmaceutical Research.

NIH/ORDA Receipt Date: 4-10-98. Not Selected for RAC Public Review: 4-30-98

Completed, long-term follow-up continued: October 1999

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**9804-244 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Cationic Liposome Complex/Plasmid DNA/Interleukin-2 cDNA/Staphylococcus Enterotoxin B (SEB)/Intratumoral Injection**

Walsh, Patrick; University of Colorado Health Sciences Center, Denver, Colorado; *A Phase I Study Using Direct Combination DNA Injections for the Immunotherapy of Metastatic Melanoma*.

NIH/ORDA Receipt Date: 4-10-98. Publicly Reviewed at the June 19, 1998 RAC meeting

Closed to enrollment, follow-up is continuing: 9-5-01

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**9804-245 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) cDNA/Aerosol Administration**

Moss, Richard; Stanford University School of Medicine, Palo Alto, California; Aitken, Moira; University of Washington Medical Center, Seattle, Washington; and Waltz, David; Harvard Medical School, Boston, Massachusetts; *A Phase I Study of Aerosolized tgAAVCF for the Treatment of Cystic Fibrosis Patients with Mild Lung Disease*. Sponsor: Targeted Genetics Corporation.

NIH/ORDA Receipt Date: 4-18-98. Not Selected for RAC Public Review: 12-3-98

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**9804-246 (Closed) Gene Therapy/Phase II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Oncogene Regulation/HER-2/neu/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intratumoral Injection**

Yoo, George H.; Wayne State University School of Medicine, Detroit, Michigan; Villaret, Douglass B.; University of Washington, Seattle, Washington; Gleich, Lyon; University of Cincinnati Medical Center, Cincinnati, Ohio; Hanna, Ehab; University of Arkansas Cancer Research Center, Little Rock, Arkansas; and Kenady, Daniel E. and Valentino, Joseph; University of Kentucky, Lexington, Kentucky; *A Multicenter Phase II Study of E1A Lipid Complex for the Intratumoral Treatment of Patients with Recurrent Head and Neck Squamous Cell Carcinoma*. Sponsor: Targeted Genetics Corporation.

NIH/ORDA Receipt Date: 4-18-98. Not Selected for RAC Public Review: 2-1-99  
Completed: 8-08-02

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**9804-247 (Closed) Gene Therapy/Phase I/Monogenic Disease/Hemophilia A/In Vitro/Electroporation/Autologous Fibroblasts/Plasmid DNA/Factor VIII cDNA/Intraperitoneal Implantation**

Roth, David A.; Beth Israel Deaconess Medical Center, Boston, Massachusetts; *A Phase I Safety Study of Autologous Transfected Human Fibroblasts Producing Human Factor VIII in Patients with Severe Hemophilia A*. Sponsor: Transkaryotic Therapies, Inc.

NIH/ORDA Receipt Date: 4-17-98. Publicly Reviewed at the June 19, 1998 RAC meeting  
Closed to enrollment: 3-26-03

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**9804-248 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Breast Cancer/Immunotherapy/In Vivo/Adenovirus/Serotype 5/B7.1 (CD80) cDNA/Intratumoral Injection**

Schuchter, Lynn; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *Phase I Trial of Therapeutic Cancer Vaccine Using Intratumoral Injections of B7-1 (H5.030CMVhB7) in Patients with Metastatic Melanoma or Metastatic Breast Cancer*.

NIH/ORDA Receipt Date: 4-23-98. Not Selected for RAC Public Review: 5-13-98

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**9804-249 (Open) Gene Therapy/Phase I/Cancer/Adenocarcinoma Expressing Carcinoembryonic Antigen (CEA)/In Vitro/Autologous T Lymphocytes/Retrovirus/anti-CEA-sFv-Zeta T Cell Receptor/Intravenous Infusion**

Junghans, Richard Paul; Beth Israel Deaconess Medical Center, Boston, Massachusetts; *Phase I Study of T Cells Modified with Chimeric AntiCEA Immunoglobulin-T Cell Receptors (IgTCR) in Adenocarcinoma*.

NIH/ORDA Receipt Date: 4-28-98. Not Selected for RAC Public Review: 5-18-98

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**9804-250 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injections**

Swisher, Steven; University of Texas M.D. Anderson Cancer Center/Texas Heart Institute, Houston, Texas; *An Efficacy Study of Adenoviral Vector Expressing Wildtype p53 (Ad5CMV-p53) Administered Intralesionally as an Adjunct to Radiation Therapy in Patients with Non-Small Cell Lung Cancer*. Sponsor: Aventis (formerly Gencell)

NIH/ORDA Receipt Date: 4-28-98. Not Selected for RAC Public Review: 5-18-98

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**9805-251 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/MUC -1/Interleukin-2/Intramuscular Injection**

Figlin, Robert; University of California at Los Angeles, Los Angeles, California; *Phase I/II Trial of Antigen-Specific Immunotherapy in MUC-1 Positive Patients with Adenocarcinoma of the Prostate Using Vaccinia Virus-MUC1-IL2 (TG 1031)*. Sponsor: Transgene, S.A.

NIH/ORDA Receipt Date: 5-1-98. Not Selected for RAC Public Review: 5-22-98  
Completed, long-term follow-up continues: 5-05-03

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**9805-252 (Open) Gene Therapy/Phase I/Cancer/Colorectal/In Vitro/Allogeneic Tumor Cells and Fibroblasts/Lethally Irradiated/Plasmid DNA/Interleukin-2 cDNA/B7.1 (CD80)/Subcutaneous Injection**

Sobol, Robert E.; Sidney Kimmel Cancer Center, San Diego, California; *A Phase I Study of Allogeneic Tumor Cells Genetically Modified to Express B7.1 (CD80) Mixed with Allogeneic Fibroblasts Genetically Modified to Secrete IL-2 in Patients with Colorectal Carcinoma.*

NIH/ORDA Receipt Date: 5-7-98. Not Selected for RAC Public Review: 5-27-98

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**9805-253 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD8+ T Cells/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous Infusion**

Scadden, David T.; Massachusetts General Hospital Cancer Center, Harvard Medical School, Boston, Massachusetts; Mitsuyasu, Ronald; University of California, Los Angeles, Los Angeles, California; and Deeks, Steven; University of California, San Francisco, San Francisco, California; *A Phase II Study of Autologous CD4-Zeta Gene-Modified T Cells in HIV Infected Patients with Undetectable Plasma Viremia on Highly Active Anti-Retroviral Drug Therapy* Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 5-14-98. Not Selected for RAC Public Review: 6-3-98  
Study closed to new accrual, follow-up is continuing: 7-13-01

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**9805-254 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/gp 100 Melanoma Antigen/Intradermal or Intramuscular Injection**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Immunization of Patients with Metastatic Melanoma Using DNA Encoding the GP100 Melanoma Antigen.* Sponsor: National Cancer Institute - Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 6-4-98. Not Selected for RAC Public Review: 6-24-98

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**9806-255 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intraperitoneal Administration**

Muller, Carolyn Y.; University of Texas Southwestern Medical School, Dallas, Texas; *Phase I Trial of Intraperitoneal Adenoviral p53 Gene Therapy in Patients with Advanced Recurrent or Persistent Ovarian Cancer.* Sponsor: National Cancer Institute - Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 6-2-98. Not Selected for RAC Public Review: 6-22-98  
Closed to accrual: 4-15-02

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**9806-256 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal and Subcutaneous Injections**

Suzuki, Tsuneo; University of Kansas Medical Center, Kansas City, Kansas; *Autologous, Irradiated, Melanoma Cells Transduced Ex Vivo with an Adenovirus Vector (Adv/GM-CSF) Expressing Granulocyte-Macrophage Colony Stimulating Factor Gene.*

NIH/ORDA Receipt Date: 6-3-98. Not Selected for RAC Public Review: 6-23-98

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**9806-257 (Open) Gene Therapy/Phase I/Cancer/Breast/Colon/Head and Neck/Soft Tissue Sarcoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/ Adenovirus/Serotype 5/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal and Subcutaneous Injections**

Suzuki, Tsuneo; University of Kansas Medical Center, Kansas City, Kansas; *Autologous, Irradiated, Cancer Cells (Breast Cancer, Colon Cancer, Head and Neck Cancer, and Soft Tissue Sarcoma) Transduced Ex Vivo with an Adenovirus Vector (Adv/GM-CSF) Expressing Granulocyte-Macrophage Colony Stimulating Factor Gene.*

NIH/ORDA Receipt Date: 6-3-98. Not Selected for RAC Public Review: 6-23-98

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**9806-258 (Closed) Gene Therapy/Phase I/ Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration**

Crystal, Ronald G.; Cornell University Medical College, New York, New York; *Phase I Study of Direct Administration of a Replication Deficient Adenovirus Vector (Ad<sub>EV</sub>VEGF121.10) Containing the VEGF121 cDNA to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease Via Minimally Invasive Surgery.* Sponsor: Parke-Davis Pharmaceutical Research.

NIH/ORDA Receipt Date: 6-8-98. Not Selected for RAC Public Review: 8-13-98  
Completed, long-term follow-up continued: October 1999

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**9806-259 (Closed) Gene Therapy/Phase II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1102/Interleukin-2 cDNA/Intratumoral Injection**

Figlin, Robert; University of California at Los Angeles, Los Angeles, California; Thompson, John, A.; University of Washington, Seattle, Washington; Galanis, Evan; Mayo Clinic, Rochester, Minnesota; and Bukowski, Ronald; Cleveland Clinic Foundation, Cleveland, Ohio; *Phase II Study of Direct Gene Transfer of IL-2 Plasmid DNA/DMRIE/DOPE Lipid Complex (Leuvectin) as an Immunotherapeutic Regimen in Patients with Metastatic Renal Cell Carcinoma*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 6-15-98. Not Selected for RAC Public Review: 7-6-98

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**9806-260 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical-1005/HLA-B7/Beta 2-Microglobulin cDNA/Concurrent Interleukin-2 Injection/Direct Intratumoral Injection**

Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; *Phase I Study of HLA-B7/β2M Plasmid DNA/DMRIE/DOPE Lipid Complex (Allovectin-7) by Direct Gene Transfer with Concurrent Low-Dose Subcutaneous IL-2 Protein Therapy as an Immunotherapeutic Regimen in Malignant Melanoma*.

NIH/ORDA Receipt Date: 6-26-98. Not Selected for RAC Public Review: 7-16-98

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**9806-261 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/Retrovirus/Transdominant Rev or Rev and Antisense Pol 1/Intravenous Infusion**

Amado, Rafael G.; University of California at Los Angeles, Los Angeles, California; Yuen, Alan R.; Stanford University Medical Center, Stanford, California; Scadden, David T.; Massachusetts General Hospital, Boston, Massachusetts; Lill, Michael; Cedars-Sinai Medical Center, Los Angeles, California; and Carabasi, Matthew; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I/II Study of the Safety and Feasibility of RevM10 or RevM10/Antisense Pol 1 Transduced Hematopoietic Stem Cells (HSC) in HIV-1 Related Non-Hodgkin's Lymphoma Patients Already Being Treated with High Dose Chemotherapy and Peripheral Blood Stem Cell Support*. Sponsor: Systemix, Inc.

NIH/ORDA Receipt Date: 6-30-98. Not Selected for RAC Public Review: 7-20-98

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**9807-262 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intraperitoneal Administration**

Wolf, Judith K.; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Study of Ad-p53 (NSC#683550) for Patients with Platinum- and Paclitaxel-Resistant Epithelial Ovarian Cancer*.

NIH/ORDA Receipt Date: 7-24-98. Not Selected for RAC Public Review: 8-13-98

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**9808-263 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection**

Lang, Frederick F., Jr. and Yung, W. K. Alfred; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; and Greenberg, Harry; University of Michigan, Ann Arbor, Michigan; *Phase I Trial of Adenovirus-Mediated Wild Type p53 Gene Therapy for Malignant Gliomas*. Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 8-13-98. Not Selected for RAC Public Review: 9-2-98

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**9808-264 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Vaccinia Virus/MUC-1/Interleukin-2/Intramuscular Injection**

Gitlitz, Barbara J.; University of California Los Angeles, Los Angeles, California; *Phase I/II Trial of Antigen-Specific Immunotherapy in MUC-1 Positive Patients with Advanced Non-Small Cell Lung Cancer Using Vaccinia-Virus-MUC1-IL2 (TG1031)*. Sponsor: Transgene, S.A.

NIH/ORDA Receipt Date: 8-27-98. Not Selected for RAC Public Review: 9-18-98

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**9809-265 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Chemoprotection/In Vitro/Peripheral Blood CD34+ Cells/Retrovirus/O<sup>6</sup>-Methylguanine DNA Methyltransferase cDNA/Intravenous Infusion**

Gerson, Stanton L.; Case Western Reserve University, Cleveland, Ohio; *Mutant MGMT Gene Transfer Into Human Hematopoietic Progenitors to Protect Hematopoiesis During O<sup>6</sup>-Benzylguanine (BG, NSC 637037) and BCNU Therapy of Advanced Solid Tumors*.

NIH/ORDA Receipt Date: 9-2-98. Not Selected for RAC Public Review: 9-23-98

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**9809-266 (Open) Gene Therapy/Phase I-II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Human Interferon-alpha cDNA/Intratumoral Injection**

McQuone, Shelly J.; The University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Multi-Center, Open-Label, Multiple Administration, Rising Dose Study of the Safety, Tolerability, and Efficacy of IFN-alpha Gene Medicine in Patients with Unresectable or Recurrent/Refractory Squamous Cell Carcinoma of the Head and Neck (SCCHN)*. Sponsor: GeneMedicine, Inc.

NIH/ORDA Receipt Date: 9-22-98. Not Selected for RAC Public Review: 3-19-99

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**9810-267 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection**

Morris, John C.; National Institutes of Health, Bethesda, Maryland; *A Phase I Study of Intralesional Administration of an Adenovirus Vector Expressing the HSV-1 Thymidine Kinase Gene (AdV.RSV-TK) in Combination with Escalating Doses of Ganciclovir in Patients with Cutaneous Metastatic Malignant Melanoma*.

NIH/ORDA Receipt Date: 10-6-98. Not Selected for RAC Public Review: 10-27-98

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**9810-268 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Irradiated/Adenovirus/Serotype 5/B7.1 (CD80) cDNA/Subcutaneous Injection**

Antonia, Scott J.; University of South Florida, Tampa, Florida; *Treatment of Patients with Stage IV Renal Cell Carcinoma with B7-1 Gene-Modified Autologous Tumor Cells and Systemic IL-2*.

NIH/ORDA Receipt Date: 10-26-98. Not Selected for RAC Public Review: 11-30-98  
Closed to new accrual: 3-29-01

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**9811-269 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Type 5/MART-1 Melanoma Antigen/Intravenous or Intradermal Injection**

Economou, James S.; UCLA Medical Center, Los Angeles, California; *A Phase I Trial Testing MART-1 Genetic Immunization in Malignant Melanoma*.

NIH/ORDA Receipt Date: 11-17-98. Not Selected for RAC Public Review: 12-8-98

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**9811-270 (Closed) Gene Therapy/Phase II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1005/HLA-B7/Beta2-Microglobulin cDNA/Direct Intratumoral Injection**

Hanna, Ehab; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Wagman, Lawrence D.; City of Hope National Medical Center, Duarte, California; Gluckman, Jack L.; University of Cincinnati Medical Center, Cincinnati, Ohio; and Wolf, Gregory T.; University of Michigan Medical Center, Ann Arbor, Michigan; *Phase II Study of the Safety, Efficacy, and Effect on Quality of Life of Allovectin-7 Immunotherapy for the Treatment of Recurrent or Persistent Squamous Cell Carcinoma of the Head and Neck*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 11-19-98. Not Selected for RAC Public Review: 2-5-99

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**9811-271 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with Moderate-Risk Critical Limb Ischemia*. Sponsor: Vascular Genetics, Inc.

NIH/ORDA Receipt Date: 11-23-98. Not Selected for RAC Public Review: 12-14-98  
Follow-up has been completed: 11-29-01

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**9811-272 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/MUC-1/Intradermal Injection**

Kufe, Donald W.; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Trial of Recombinant Vaccinia Virus that Expresses DF3/MUC1 in Patients with Metastatic Adenocarcinoma of the Breast*.

NIH/ORDA Receipt Date: 11-23-98. Not Selected for RAC Public Review: 12-24-98

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**9812-273 (Open) Gene Therapy/Phase I-II/Infectious Diseases/Human Immunodeficiency Virus-1 (HIV-1)/In Vitro/Immunotherapy/Autologous CD8+ HIV-Specific T Cells/Retrovirus/Neomycin Phosphotransferase Gene/Intravenous Infusion**

Riddell, Stanley R.; Fred Hutchinson Cancer Research Center, Seattle, Washington; *The Safety and Antiviral Efficacy of Cellular Adoptive Immunotherapy with Autologous CD8+ HIV-Specific Cytotoxic T Cells Combined with Interleukin-2 for HIV Seropositive Individuals.*

NIH/ORDA Receipt Date: 12-3-98. Not Selected for RAC Public Review: 1-5-99

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**9812-274 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Fibroblast Growth Factor (FGF) cDNA/Intramuscular Injection**

Comerota, Anthony J.; Temple University School of Medicine, Philadelphia, Pennsylvania; Laird, John R.; Washington Hospital Center, Washington, D.C.; Sequeira, Rafael F.; University of Miami, School of Medicine, Miami, Florida; Henry, Timothy; Hennepin County Medical Center, Minneapolis, Minnesota; and Chronos, Nicholas; Atlanta Cardiology Group, Atlanta, Georgia; *A Phase I, Multi-Center, Open Label, Safety and Tolerability Study of Increasing Single Dose of NV1FGF Administered by Intra-Muscular Injection in Patients with Severe Peripheral Artery Occlusive Disease.* Sponsor: Aventis (formerly Gencell).

NIH/ORDA Receipt Date: 12-17-98. Not Selected for RAC Public Review: 2-4-99

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**9812-275 (Open) Gene Therapy/Phase I/Cancer/Advanced Malignancies/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intravenous Injection**

Eckhardt, S. Gail; Institute for Drug Development, Cancer Therapy and Research Center, San Antonio, Texas; *A Pharmacokinetic, Safety and Tolerability Study of Intravenous INGN in Patients with Advanced Cancer.* Sponsor: Introgen Therapeutics, Inc.

NIH/ORDA Receipt Date: 12-18-98. Not Selected for RAC Public Review: 5-13-99

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**9812-276 (Open) Gene Therapy/Phase I/Cancer/Prostate/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Valacyclovir/Intratunoral Injection**

Gardner, Thomas A. and Chung, Leland W. K.; University of Virginia Health, Charlottesville, Virginia; *Phase I Study of Ad-OC-TK Plus Valacyclovir for the Treatment of Metastatic or Recurrent Prostate Cancer.*

NIH/ORDA Receipt Date: 12-23-98. Not Selected for RAC Public Review: 1-14-99

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**9812-277 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/Retrovirus/Transdominant Rev/Antisense Pol 1/Intravenous Infusion**

Amado, Rafael G.; University of California, Los Angeles, Los Angeles, California; Carabasi, Matthew; University of Alabama at Birmingham, Birmingham, Alabama; Swindells, Susan; University of Nebraska Medical Center, Omaha, Nebraska; and Scadden, David T.; Massachusetts General Hospital, Boston, Massachusetts; *A Phase I/II Study in HIV-1 Infected Patients Infused with CD34+Thy1+ Hematopoietic Stem Cells (HSC) from G-CSF Mobilized Peripheral Blood Retrovirally Transduced with RevM10 or RevM10/Antisense Pol1.* Sponsor: Systemix, Inc.

NIH/ORDA Receipt Date: 12-28-98. Not Selected for RAC Public Review: 1-19-99

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**9901-278 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/MART-1 Melanoma Antigen/Intramuscular Injection**

Conry, Robert M.; University of Alabama at Birmingham, Birmingham, Alabama; *Phase I Dose Escalation Trial of Polynucleotide Immunization with Plasmid DNA Encoding MART-1 (Melanoma Antigen Recognized by T Cells-1) in Patients with Resected Melanoma at Significant Risk for Relapse.*

NIH/ORDA Receipt Date: 1-4-99. Not Selected for RAC Public Review: 1-25-99

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**9901-279 (Open) Gene Therapy/Phase I/Monogenic Disease/Hemophilia B/In Vivo/Adeno-Associated Virus/Factor IX Gene/Intramuscular Injection**

Manno, Catherine S.; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Phase I Safety Study in Patients with Severe Hemophilia B (Factor IX Deficiency) Using Adeno-Associated Viral Vector to Deliver the Gene for Human Factor IX to Skeletal Muscle.* Sponsor: Avigen.

NIH/ORDA Receipt Date: 1-7-99. Publicly Reviewed at the March 12, 1999 RAC meeting

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**9901-280 (Closed) Gene Therapy/Phase II-III/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intraperitoneal Administration**

Buller, Richard; The University of Iowa Hospitals and Clinics, Iowa City, Iowa; Carson, Linda F.; University of Minnesota, Minneapolis, Minnesota; Weisberg, Tracey; Maine Center for Cancer Medicine, Scarborough, Maine; Christopherson, Wayne A.; Mercy Hospital of Pittsburgh, Pittsburgh, Pennsylvania; Molpus, Kelly; University of Nebraska Medical Center, Omaha, Nebraska; Davidson, Susan A.; University of Colorado Health Sciences Center, Denver, Colorado; Gutheil, John C.; Sharp HealthCare, Sidney Kimmel Cancer Center, San Diego, California; Bloss, Jeffrey D.; University of Missouri, Columbia, Missouri; Blum, Ronald; Beth Israel Medical Center, New York, New York; Puls, Larry E.; Greenville Hospital System, Greenville, South Carolina; Teng, Nelson Nan-Hsiung; Stanford University School of Medicine, Stanford, California; Pergram, Mark D.; University of California, Los Angeles, Los Angeles, California; Ueland, Frederick; University of Kentucky Medical Center, Lexington, Kentucky; Rodriguez, Michael; University Hospitals of Cleveland, Cleveland, Ohio; Malfetano, John H.; Albany Medical College, Albany, New York; Edwards, Robert P.; University of Pittsburgh, Pittsburgh, Pennsylvania; Rader, Janet; Washington University, Saint Louis, Missouri; Benigno, Benedict B.; Northside Hospital, Atlanta, Georgia; Lucci, Joseph T.; University of Texas Medical Branch, Galveston, Texas; Delmore, James E.; University of Kansas School of Medicine, Wesley Medical Center, Wichita, Kansas; Smith, Harriet O.; University of New Mexico School of Medicine, Albuquerque, New Mexico; Bristow, Robert E.; Johns Hopkins School of Medicine, Baltimore, Maryland; Abbas, Fouad; Sinai Hospital of Baltimore, Baltimore, Maryland; Fort, Giles; Woman's Hospital, Baton Rouge, Louisiana; Berchuck, Andrew; Duke University Medical Center; Coleman, Robert; University of Texas Southwestern Medical Center, Dallas, Texas; Rocereto, Thomas; University of Medicine and Dentistry of New Jersey, Robert Wood Johnson Medical School, Camden, New Jersey; Hall, James; Carolinas Medical Center, Charlotte, North Carolina; Holloway, Robert; Walt Disney Memorial Cancer Institute, Orlando, Florida; Garcia, Agustin; University of Southern California, Norris Cancer Hospital, Los Angeles, California; Lentz, Samuel Wake Forest University School of Medicine, Winston-Salem, North Carolina; Swensen, Ron; Loma Linda University Cancer Institute, Loma Linda, California; Horowitz, Ira; Emory University School of Medicine, Atlanta, Georgia; Kline, Richard and Burroff, Janet; Alton Ochsner Medical Foundation, New Orleans, Louisiana; Scudder, Sidney; University of California, Davis, Sacramento, California; Noubisi, Boniface; University of Florida, Gainesville, Florida; and Celano, Paul; Greater Baltimore Medical Center, Baltimore, Maryland; *A Phase II/III Trial of Chemotherapy Alone Versus Chemotherapy Plus SCH 58500 in Newly Diagnosed Stage III Ovarian and Primary Peritoneal Cancer Patients with  $\geq 0.5$  cm and  $\leq 2$  cm Residual Disease Following Surgery*. Sponsor: Schering Corporation

NIH/ORDA Receipt Date: 1-12-99. Not Selected for RAC Public Review: 6-2-99  
Closed to enrollment: 3-30-01

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**9901-281 (Closed) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Type 5/MART-1 Melanoma Antigen/gp 100 Melanoma Antigen/Subcutaneous Injection**

Haluska, Frank; Harvard Medical School, Boston, Massachusetts and Nemunaitis, John J.; US Oncology, Dallas, Texas; *Phase I/II Trial of the Safety, Immunogenicity, and Efficacy of Autologous Dendritic Cells Transduced with Adenoviruses Encoding the MART-1 and gp100 Melanoma Antigens Administered With or Without Low Dose Recombinant Interleukin-2 (rIL-2) in Patients with Stage IV Melanoma*. Sponsor: Genzyme Molecular Oncology.

NIH/ORDA Receipt Date: 1-12-99. Not Selected for RAC Public Review: 3-30-99  
Closed: 6-17-02

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**9901-282 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen/Intramuscular Injection**

Eder, Joseph Paul; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase II Randomized Trial of Recombinant Fowlpox and Recombinant Vaccinia Virus Expressing PSA in Patients with Adenocarcinoma of the Prostate*. Sponsor: National Cancer Institute-Cancer Therapy Evaluation Program (NCI-CTEP).

NIH/ORDA Receipt Date: 1-12-99. Not Selected for RAC Public Review: 1-24-00

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**9901-283 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection**

Small, Eric J.; University of California, San Francisco, San Francisco, California; *Phase I/II Study of a Prime-Boost Schedule of Human GM-CSF Gene Transduced Irradiated Prostate Allogeneic Cancer Cell Vaccines (Allogeneic Prostate GVAX<sup>TM</sup>) in Hormone-Naive Prostate Cancer Patients*. Sponsor: Cell Genesys

NIH/ORDA Receipt Date: 1-22-99. Not Selected for RAC Public Review: 2-11-99  
Notification from sponsor that trial is closed: 4-09-01

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**9902-284 (Open) Gene Therapy/Phase I/Monogenic Disease/Hemophilia A/In Vivo/Retrovirus/Factor VIII cDNA/Intravenous Infusion**

Ragni, Margret V.; University of Pittsburgh, Pittsburgh, Pennsylvania; Lusher, Jeanne M.; Children's Hospital of Michigan, Detroit, Michigan; Powell, Jerry S.; University of California, Davis, Medical Center, Sacramento, California; White, Gilbert; University of North Carolina School of Medicine, Chapel Hill, North Carolina and Ewenstein, Bruce M.; Brigham and Women's Hospital, Boston, Massachusetts; *Phase I Multi-Center, Single Treatment Dose Escalation Study of Factor VIII Vector [HFVIII(V)] for Treatment of Severe Hemophilia A*. Sponsor: Chiron Corporation

NIH/ORDA Receipt Date: 2-5-99. Publicly Reviewed at the September 3, 1999 RAC meeting

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**9902-285 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/In Vivo/Cationic Liposome Complex with DC-Chol/Epidermal Growth Factor Receptor Antisense/Intratumoral Injection**

Grandis, Jennifer Rubin; University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *A Phase I Trial of Intratumoral Antisense EGFR DNA and DC-Chol Liposomes in Advanced Oral Squamous Cell Carcinoma.*

NIH/ORDA Receipt Date: 2-12-99. Not Selected for RAC Public Review: 3-5-99

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**9902-286 (Open) Gene Therapy/Phase I/Cancer/Lung, Head and Neck/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1005/HLA-B7/Beta 2-Microglobulin cDNA/Concurrent Interleukin-2 Injection/Direct Intratumoral Injection**

Stopeck, Alison; Arizona Cancer Center, University of Arizona, Tucson, Arizona; *Phase I Study of HLA-B7/beta2M Plasmid DNA/DMRIE/DOPE Lipid Complex (Allovectin-7) by Direct Gene Transfer with Concurrent Low-Dose Subcutaneous IL-2 Protein Therapy as an Immunotherapeutic Regimen in Lung and Head and Neck Cancers.* Sponsor: Vical Inc.

NIH/ORDA Receipt Date: 2-16-99. Not Selected for RAC Public Review: 3-8-99

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**9902-287 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Bronchoalveolar Lavage**

Schiller, Joan; University of Wisconsin, Madison, Wisconsin; and Carbone, David, P.; Vanderbilt University Medical Center, Nashville, Tennessee; *Phase I Pilot Trial of Adenovirus p53 in Bronchioloalveolar Cell Lung Carcinoma (BAC) Administered by Bronchoalveolar Lavage.* Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 2-16-99. Not Selected for RAC Public Review: 3-25-99

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**9902-288 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection (Endobronchial or Percutaneous)**

Schiller, Joan; University of Wisconsin, Madison, Wisconsin; *Phase I Pilot Trial of Adenovirus p53 and Radiotherapy on Non-Small Cell Lung Cancer.* Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 2-18-99. Not Selected for RAC Public Review: 6-24-99

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**9902-289 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cationic Liposome Complex/Alpha-1 Antitrypsin cDNA/Intranasal Administration**

Brigham, Kenneth L.; Vanderbilt University School of Medicine, Nashville, Tennessee; *Expression of an Exogenously Delivered Human Alpha-1 Antitrypsin Gene in Nasal Epithelium of Patients with Cystic Fibrosis.*

NIH/ORDA Receipt Date: 2-19-99. Not Selected for RAC Public Review: 4-2-99  
Closed to enrollment: 5-02-02

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**9902-290 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Particle Mediated Gene Transfer (Accel<sup>®</sup>)/Plasmid DNA/gp 100 cDNA/Granulocyte-Macrophage Colony Stimulating Factor cDNA**

Albertini, Mark R.; University of Wisconsin, Madison, Wisconsin; *Phase I Trial of Immunization Using Particle-Mediated Transfer of Genes for GP-100 and GM-CSF into Uninvolved Skin of Patients with Melanoma.*

NIH/ORDA Receipt Date: 2-22-99. Not Selected for RAC Public Review: 3-15-99  
3-29-00: Closed to accrual and treatment; follow-up will continue

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**9902-291 (Open) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Fanconi Anemia Complementation Group A cDNA/Intravenous**

Walsh, Christopher E.; The University of North Carolina at Chapel Hill, Chapel Hill, North Carolina; *Retroviral-Mediated Gene Transfer of the Fanconi Anemia Group A Gene into Hematopoietic Progenitor Cells of Group A Patients.*

NIH/ORDA Receipt Date: 2-22-99. Not Selected for RAC Public Review: 3-15-99

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**9902-292 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/gp 100 Melanoma Antigen/Intramuscular or Intravenous Injection**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Immunization of Patients with Metastatic Melanoma Using a Recombinant Fowlpox Virus Encoding a GP 100 Peptide Preceded by an Endoplasmic Reticulum Insertion Signal Sequence*. Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 2-24-99. Not Selected for RAC Public Review: 3-22-99

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**9902-293 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen/Intramuscular or Intradermal Injection**

Kaufman, Howard; Albert Einstein College of Medicine, Bronx, New York; *Phase II Randomized Study of Vaccine Treatment of Advanced Prostate Cancer*. Sponsor: Eastern Cooperative Oncology Group

NIH/ORDA Receipt Date: 2-24-99. Not Selected for RAC Public Review: 8-13-99

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**9902-294 (Closed) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Administration**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts, Henry, Timothy D.; Hennepin County Medical Center, Minneapolis, Minnesota and Schatz, Richard A.; Scripps Clinic, La Jolla, California; *A Multicenter, Open-Label, Dose-Escalating Study of Intramyocardial Vascular Endothelial Growth Factor 2 (VEGF-2) Gene Therapy in Refractory Patients with Stable Exertional Angina Who Are Not Candidates for Revascularization Procedures*. Sponsor: Coraetus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 2-26-99. Not Selected for RAC Public Review: 6-11-99

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**9903-295 (Withdrawn from RAC Review) Gene Therapy/Phase I/Monogenic Disease/Gyrate Atrophy/In Vitro/Autologous Keratinocytes/Retrovirus/Ornithine Aminotransferase (OAT) cDNA/Skin Patch Administration**

Nussenblatt, Robert B.; National Institutes of Health, Bethesda, Maryland; *Phase I Study in the Safety and Efficacy of Transduced Keratinocytes for Possible Treatment of Gyrate Atrophy*.

NIH/ORDA Receipt Date: 3-4-99. Withdrawn from RAC review: 9-18-00

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**9903-296 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Gamma Interferon cDNA/Intratatumoral Injection**

Rosenblatt, Joseph D.; University of Rochester Medical Center, Rochester, New York; *Phase I Trial of Immunotherapy with Adenovirus-Interferon-Gamma (TG1041) in Patients with Malignant Melanoma*. Sponsor: Transgene, Inc.

NIH/ORDA Receipt Date: 3-10-99. Not Selected for RAC Public Review: 3-30-99  
Closed by sponsor to further enrollment: 06-29-01

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**9903-297 (Closed) Gene Marking/Autoimmune Disease/Multiple Sclerosis/In Vitro/CD34+ Autologous Peripheral Blood/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Infusion**

Krance, Robert; Baylor College of Medicine, Houston, Texas; *Intensive Immunosuppression Followed by Rescue with CD34 Selected, T Cell Depleted, Leukopheresis Products in Patients with Multiple Sclerosis*.

NIH/ORDA Receipt Date: 3-24-99. Not Selected for RAC Public Review: 4-21-99  
Gene marking portion of this study has been removed: 3-26-03

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**9903-298 (Open) Gene Therapy/Phase II/Cancer/Ovarian/Pro-Drug/In Vivo/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intraperitoneal/Catheter**

Link, Charles J. and Morrmann, Donald; Human Gene Therapy Research Institute, Des Moines, Iowa; *A Phase II Trial of In Vivo Gene Therapy with the Herpes Simplex Thymidine Kinase for the Treatment of Ovarian Cancer*.

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99

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**9903-299 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; Baumgartner, Iris; Bern University, Bern Switzerland and Olin, Jeffrey Wayne; Cleveland Clinic Foundation, Cleveland, Ohio; *A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with Moderate-Risk Critical Limb Ischemia*. Sponsor: Coraetus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99  
Closed: 2-11-00

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**9903-300 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with High-Risk Critical Limb Ischemia*.

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99  
Follow-up has been completed: 11-29-01

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**9903-301 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; Baumgartner, Iris; Bern University, Bern Switzerland and Olin, Jeffrey Wayne; Cleveland Clinic Foundation, Cleveland, Ohio; *A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with High-Risk Critical Limb Ischemia*. Sponsor: Coraetus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99  
Closed: 2-11-00

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**9903-302 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Open-Label, Rescue-Therapy Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with Moderate-Risk or High-Risk Critical Limb Ischemia*. Sponsor: Coraetus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99  
Follow-up is complete: 11-29-01

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**9903-303 (Closed) Gene Marking/Cancer/Neuroblastoma/Sarcoma/Retinoblastoma/In Vitro/CD34+ Autologous Peripheral Blood or Bone Marrow/Dihydrofolate Reductase cDNA/Intravenous Infusion**

Cunningham, John M.; St. Jude Children's Research Hospital, Memphis, Tennessee; *Tumor Purging of Autologous Stem Cell Grafts in Children with High-Risk Solid Tumors: Transplantation of Retrovirally Marked Stem Cell Grafts Purified by CD34+ Antibody Selection and High-Speed Cell Sorting*.

NIH/ORDA Receipt Date: 3-29-99. Not Selected for RAC Public Review: 5-18-99  
Closed: 11-4-02

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**9904-304 (Closed) Gene Therapy/Phase I/Cancer/Retinoblastoma/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection (Intraocular Tumor)**

Hurwitz, Richard L.; Baylor College of Medicine, Houston, Texas; *Pediatric Phase I Study of AdV/RSV-TK Followed by Ganciclovir for Retinoblastoma*

NIH/ORDA Receipt Date: 4-1-99. Publicly Reviewed at the June 14, 1999 RAC meeting  
Closed: 7-15-04

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**9904-305 (Open) Gene Therapy/Phase I/Cancer/Breast/Tumor Suppressor Gene/In Vitro/Autologous CD34+ Cells/Adenovirus/Serotype 5/p53 cDNA/Intravenous Infusion**

Baynes, Roy D.; Karmanos Cancer Institute, Wayne State University, Detroit, Michigan; *A Phase I Study of Infused Mobilized, Autologous Peripheral Blood Progenitor Cells, Which Have Been Incubated with a Recombinant Adenovirus-Wild-Type p53 Construct (SCH 58500) to Purge Any Contaminating Breast Cancer Cells, As Stem Cell Support After High-Dose Chemotherapy in Patients with Breast Cancer Metastatic to Bone and Bone Marrow.*

NIH/ORDA Receipt Date: 4-5-99. Not Selected for RAC Public Review: 5-3-99

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**9904-306 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Prostate Specific Antigen/Intravenous**

Vieweg, Johannes; Duke University Medical Center, Durham North Carolina; *Safety and Feasibility Study of Active Immunotherapy in Patients with Hormone Refractory Prostate Cancer Using Autologous Dendritic Cells Pulsed with RNA Encoding Prostate Specific Antigen, PSA*

NIH/ORDA Receipt Date: 4-6-99. Not Selected for RAC Public Review: 4-26-99  
Closed to enrollment: 2-13-03

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**9904-307 (Closed) Gene Therapy/Phase I/Cancer/Cervical/Immunotherapy/In Vivo/Vaccinia Virus/Human Papilloma Virus E6 and E7/Interleukin-2/Intramuscular Injection**

Kaufman, Raymond H.; Baylor College of Medicine, Houston, Texas; *Phase I Trial of Immunotherapy with MVA-HPV-IL2 (TG4001) in Women with Cervical Intraepithelial Neoplasia (CIN) Grade 3.* Sponsor: Transgene, Inc.

NIH/ORDA Receipt Date: 4-8-99. Not Selected for RAC Public Review: 4-26-99

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**9904-308 (Closed) Gene Therapy/Phase I/Cancer/Leukemia/Adoptive Immunotherapy/In Vitro/Donor CD8+ Lymphocytes/Retrovirus/Hygromycin Phosphotransferase-Herpes Simplex Thymidine Kinase Fusion Gene/Intravenous Infusion**

Warren, Edus; Fred Hutchinson Cancer Research Center, Seattle, Washington; *Phase I Study of Adoptive Immunotherapy with Gene-Modified and Unmodified CD8+ Minor Histocompatibility (H) Antigen-Specific CTL Clones for Patients with Relapse of AML or ALL After Allogeneic Hematopoietic Stem Cell Transplant.*

NIH/ORDA Receipt Date: 4-13-99. Not Selected for RAC Public Review: 5-3-99  
Participants will no longer receive gene-modified cells: 10-27-01

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**9904-309 (Closed) Gene Therapy/Phase I/Cancer/Cervical Cancer/Immunotherapy/In Vivo/Vaccinia Virus/Human Papilloma Virus E6 and E7/Interleukin-2/Intramuscular Injection**

Goff, Barbara A.; University of Washington School of Medicine, Seattle, Washington; *Phase I Trial of Immunotherapy with MVA-HPV-IL2 (TG4001) in Women with Advanced Cervical Carcinoma.* Sponsor: Transgene, Inc.

NIH/ORDA Receipt Date: 4-22-99. Not Selected for RAC Public Review: 5-12-99

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**9904-310 (Closed) Gene Marking/Osteodysplasia/In Vitro/Stromal Cells for Donor Bone Marrow/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Infusion**

Horwitz, Edwin M.; St. Jude Children's Research Hospital, Memphis, Tennessee; *Stromal Therapy of Osteodysplasia After Allogeneic Bone Marrow Transplantation: A Phase I Study.*

NIH/ORDA Receipt Date: 4-22-99. Not Selected for RAC Public Review: 5-12-99  
Closed to accrual, follow-up continues: 5-03-02

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**9904-311 (Open) Gene Marking/Cancer/Neuroblastoma/In Vitro/Autologous Cytotoxic T-Lymphocytes from Peripheral Blood/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Infusion**

Nuchtern, Jed; Baylor College of Medicine, Houston, Texas; *Administration of Neomycin Resistance Gene Marked Neuroblastoma Specific Cytotoxic T-Lymphocytes to Patients with Relapsed/Resistant Neuroblastoma.*

NIH/ORDA Receipt Date: 4-30-99. Not Selected for RAC Public Review: 5-20-99

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**9905-312 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1102/Leuvectin/Interleukin-2 cDNA/Intratumoral Injection**

Belldegrun, Arie; University of California, Los Angeles, Los Angeles, California; Klein, Eric; Cleveland Clinic Foundation, Cleveland, Ohio; Corman, John; VA Puget Sound Health Care System, Seattle, Washington; and Moul, Judd; Walter Reed Army Medical Center, Washington, DC; *Phase II Study Evaluating the Safety and Efficacy of Neoadjuvant Leuvectin Immunotherapy for the Treatment of Prostate Cancer*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 5-7-99. Not Selected for RAC Public Review: 5-27-99  
Closed: 6-27-03; follow-up is continuing

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**9905-313 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/Vaccinia Virus/Tyrosinase cDNA/Intramuscular Injection**

Topalian, Suzanne L.; National Institutes of Health, Bethesda, Maryland; *Immunization of Patients with Metastatic Melanoma Using Recombinant Fowlpox and Vaccinia Viruses Encoding the Tyrosinase Antigen*.

NIH/ORDA Receipt Date: 5-11-99. Not Selected for RAC Public Review: 6-1-99

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**9905-314 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Vaccinia Virus/B7.1 (CD80)/Intratumoral Injection**

Kaufman, Howard L.; Columbia University, New York, New York; *A Phase I Trial of Intralesional RV-B7.1 Vaccine in the Treatment of Malignant Melanoma*. Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 5-12-99. Not Selected for RAC Public Review: 7-23-99  
Closed: 5-15-02; follow-up is continuing

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**9905-315 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection**

Small, Eric J.; University of California, San Francisco, San Francisco, California and Smith, David C.; University of Michigan, Ann Arbor, Michigan; *A Phase I/II Study of a Prime-Boost Schedule of Human GM-CSF Gene Transduced Irradiated Prostate Allogeneic Cancer Vaccine (Allogeneic Prostate GVAX™) in Hormone-Refractory Prostate Cancer (G9803)*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 5-14-99. Not Selected for RAC Public Review: 6-4-99  
Notification from sponsor that trial is closed: 4-09-01

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**9905-316 (Closed) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Percutaneous Cardiac Catheterization**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *Multicenter, Randomized, Single-Blind, Placebo-Controlled, Dose-Escalating Study of Intramyocardial Vascular Endothelial Growth Factor 2 (VEGF2) Gene Therapy Administered Using Percutaneous Cardiac Catheterization in Patients with Refractory and Stable Exertional Angina Who Are Not Candidates for Revascularization Procedures*. Sponsor: Coraetus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 5-21-99. Not Selected for RAC Public Review: 8-30-99  
Follow-up is complete: 11-29-01

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**9905-317 (Open) Gene Therapy/Phase I/Monogenic Disease/Muscular Dystrophy/In Vivo/Adeno-Associated Virus/  $\alpha$ ,  $\beta$ ,  $\gamma$ ,  $\Delta$ -Sarcoglycan cDNA/Intramuscular Injection**

Mendell, Jerry; Ohio State University, Columbus, Ohio; *Phase I Clinical Trial Utilizing Gene Therapy for Limb Girdle Muscular Dystrophy:  $\alpha$ ,  $\beta$ ,  $\gamma$  or  $\Delta$ -Sarcoglycan Gene Delivered with Intramuscular Instillations of Adeno-Associated Vectors*.

NIH/ORDA Receipt Date: 5-26-99. Publicly Reviewed at the September 2, 1999 RAC meeting

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**9905-318 (Closed) Gene Therapy/Phase II/Cancer/Colon/Hepatic Metastasis/Tumor Suppressor Gene/n Vivo/Adenovirus/Serotype 5/p53 cDNA/Intrahepatic/Hepatic Artery/Bolus Infusion**

Venook, Alan P. and Warren, Robert S. Warren; University of California, San Francisco, San Francisco, California; Lenz, Heinz-Josef; University of Southern California, Los Angeles, California; Ravikumar, Thanjavur S.; Montefiore Medical Center, Bronx, New York; Kardinal, Carl; Alton Ochsner Medical Foundation, New Orleans, Louisiana; Roh, Mark S.; Allegheny General Hospital, Pittsburgh, Pennsylvania; Kemeny, Margaret; Stony Brook University Hospital, Stony Brook, New York; Gold, Philip J.; University of Washington, Seattle, Washington; Staley III, Charles; Emory University School of Medicine, Atlanta, Georgia; McMasters, Kelly M.; University of Louisville, Louisville, Kentucky; Elias, Laurence; University of New Mexico School of Medicine, Albuquerque, New Mexico; and Amado, Rafael G.; University of California, Los Angeles, Los Angeles, California; *A Phase II Study of SCH 58500 in Combination with Chemotherapy Alone in Patients with Colorectal Cancer Metastatic to the Liver*. Sponsor: Schering Corporation.

NIH/ORDA Receipt Date: 5-26-99. Not Selected for RAC Public Review: 6-16-99  
Notification from sponsor that study is closed to new enrollment at all sites: 3-29-01

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**9905-319 (Open) Gene Therapy/Phase I/Cancer/Acute Leukemia/Immunotherapy/In Vitro/Autologous Bone Marrow Fibroblasts/Lethally Irradiated/Adenovirus/Serotype 5/Interleukin-2 cDNA/CD40 Ligand cDNA/subcutaneous Injection**

Brenner, Malcolm; Baylor College of Medicine, Texas Children's Hospital, Baylor College of Medicine, Houston, Texas; *Treatment of High Risk Acute Leukemia with CD40 Ligand and IL-2 Gene Modified Autologous Bone Marrow Fibroblasts and Tumor Cells*.

NIH/ORDA Receipt Date: 5-26-99. Not Selected for RAC Public Review: 6-16-99

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**9905-320 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Carcinoembryonic Antigen/Intravenous**

Lyerly, H. Kim; Duke University Medical Center, Durham, North Carolina; *Pilot Study of CEA RNA-Loaded, FLT3 Ligand-Mobilized Peripheral Blood Antigen Presenting Cells for Patients with Metastatic Malignancies Expressing CEA*.

NIH/ORDA Receipt Date: 5-26-99. Not Selected for RAC Public Review: 9-23-99

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**9906-321 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/E. coli Cytosine Deaminase cDNA/5-Fluorocytosine/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection**

Kim, Jae Ho; Henry Ford Health System, Detroit, Michigan; *A Phase I Study of E1B-Attenuated Replication Competent Adenovirus Vector-Mediated Intratumoral Administration of the E. coli Cytosine Deaminase/HSV-1 Thymidine Kinase Fusion Gene in Conjunction with Two Prodrugs, 5-Fluorocytosine and Ganciclovir for Patients with Local Recurrence of Prostate Cancer after Radiation Therapy*.

NIH/ORDA Receipt Date: 6-9-99. Not Selected for RAC Public Review: 6-29-99

Study is completed: 7-3-02

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**9906-322 (Closed) Gene Therapy/Phase I/Alzheimer's Disease/In Vitro/Autologous Fibroblasts/Retrovirus/Nerve Growth Factor cDNA/Intracerebral Implantation**

Tuszynski, Mark H.; University of California, San Diego, La Jolla, California; *A Phase I Study of NGF Ex Vivo Gene Therapy for Alzheimer's Disease*

NIH/ORDA Receipt Date: 6-15-99. Publicly Reviewed at the December 1999 RAC meeting.  
Closed to enrollment: 6-18-02

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**9906-323 (Open) Gene Therapy/Phase II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Cationic Liposome Complex/DOTMA-Cholesterol/Interleukin-2 cDNA/Intratumoral Injection**

Zarrabi, M. H.; Veterans Affairs Medical Center, Northport, New York; Biel, Merrill A.; Ear, Nose & Throat SpecialtyCare of Minnesota, P.A., Minneapolis, Minnesota; Krasnow, Steven; Veterans Affairs Medical Center, Washington, D.C.; Cornett, Patricia; University of California, San Francisco/Veterans Affairs Medical Center, San Francisco, California; Robbins, K. Thomas; University of Tennessee, Memphis, Tennessee; O'Malley, Bert W.; University of Maryland School of Medicine, Baltimore, Maryland; Kabbavar, Fairouz; University of California, Los Angeles, Los Angeles, California; McCaffery, Thomas; University of South Florida, Tampa, Florida; and Cordero, Joe; Texas Tech University, Lubbock, Texas; *A Multi-Center, Open-Label, Study of the Safety and Efficacy of Multiple Intratumoral Injections of hIL-2 Plasmid (1.8 mg) Formulated with DOTMA/Cholesterol [Ratio 1:0.5 (-/+)] Liposomes in Patients with Unresectable or Recurrent/Refractory Squamous Cell Carcinoma of the Head and Neck*. Sponsor: Valentis, Inc.

NIH/ORDA Receipt Date: 6-17-99. Not Selected for RAC Public Review: 7-8-99

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**9906-324 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection**

Butler, E. Brian and Aguilar-Cordova, Estuardo; Baylor College of Medicine, Houston, Texas; *Phase I-II Study Evaluating HSV-tk + Valacyclovir Gene Therapy in Combination with Radiotherapy for Prostate Cancer.*

NIH/ORDA Receipt Date: 6-22-99. Not Selected for RAC Public Review: 7-13-99

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**9906-325 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Interferon-Beta cDNA/Stereotactic Injection**

Eck, Stephen L.; University of Pennsylvania, Philadelphia, Pennsylvania; *Treatment of Recurrent or Progressive Malignant Glioma with a Recombinant Adenovirus Expressing Human Interferon-Beta (H5.010CMVhIFN-β): A Phase I Trial.*

NIH/ORDA Receipt Date: 6-30-99. Not Selected for RAC Public Review: 7-21-99

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**9906-326 (Closed) Gene Therapy/Phase I/Cancer/Skin Metastasis/Immunotherapy/In Vivo/Plasmid DNA/Interleukin-12 cDNA/Intratumoral Injection**

Mahvi, David M.; University of Wisconsin, Madison, Wisconsin; *Treatment of Spontaneous Tumor Metastases with IL-12 DNA: A Phase IB Trial.*

NIH/ORDA Receipt Date: 6-30-99. Not Selected for RAC Public Review: 7-21-99

Closed to accrual, follow-up continues: 10-28-03

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**9907-327 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1α/VP16 cDNA/Intramuscular Injection**

Losordo, Douglas W.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; Chronos, Nicholas; Atlanta Cardiology Group, Saint Joseph's Hospital, Atlanta, Georgia; Deitcher, Steven; Cleveland Clinic Foundation, Cleveland, Ohio; Rajagopalan, Sanjay; University of Michigan; and Laird, John; Washington Hospital Center, Washington, DC; *A Phase I Double-Blind, Placebo Controlled, Escalating Dose, Multi-Center Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administered by Intramuscular Injection to Patients with Critical Limb Ischemia Who are Not Candidates for Surgical or Percutaneous Revascularization.* Sponsor: Genzyme Corporation.

NIH/ORDA Receipt Date: 7-6-99. Not Selected for RAC Public Review: 10-5-99

Study completed: 11-25-03

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**9907-328 (Open) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1α/VP16 cDNA/Intramuscular Injection**

Losordo, Douglas W.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; Chronos, Nicholas; Atlanta Cardiology Group, Saint Joseph's Hospital, Atlanta, Georgia; Deitcher, Steven; Cleveland Clinic Foundation, Cleveland, Ohio; Rajagopalan, Sanjay; University of Michigan; and Laird, John; Washington Hospital Center, Washington, DC; *A Phase I, Open-Label, Multi-Center Extension Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administered by Intramuscular Injection to Patients with Critical Limb Ischemia Who are Not Candidates for Surgical or Percutaneous Revascularization.* Sponsor: Genzyme Corporation.

NIH/ORDA Receipt Date: 7-6-99. Not Selected for RAC Public Review: 10-5-99

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**9907-329 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1α/VP16 cDNA/Intramuscular Injection**

Losordo, Douglas W.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; Chronos, Nicholas; Atlanta Cardiology Group, Saint Joseph's Hospital, Atlanta, Georgia; Deitcher, Steven; Cleveland Clinic Foundation, Cleveland, Ohio; Rajagopalan, Sanjay; University of Michigan; and Laird, John; Washington Hospital Center, Washington, DC; *A Phase I, Open-Label, Escalating Dose, Multi-Center Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administered by Intramuscular Injection to Patients with Critical Limb Ischemia Who are Not Candidates for Surgical or Percutaneous Revascularization.* Sponsor: Genzyme Corporation.

NIH/ORDA Receipt Date: 7-6-99. Not Selected for RAC Public Review: 10-5-99

Enrollment complete: 11-25-03

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**9907-330 (Closed) Gene Therapy/Phase I/Cancer/CD20+ Lymphoma/In Vitro/Autologous T Lymphocytes/Plasmid DNA/Electroporation/CD20-Specific scFvFc-Zeta T Cell Receptor/Intravenous Infusion**

Jensen, Michael; City of Hope National Medical Center, Duarte, California; *Pilot Phase I Study to Evaluate the Safety of Cellular Immunotherapy Using Genetically Modified Autologous CD20-Specific CD8+ T Cell Clones for Patients with Recurrent/Refractory CD20+ Lymphoma Undergoing Autologous Peripheral Blood Stem Cell Transplantation.*

NIH/ORDA Receipt Date: 7-8-99. Not Selected for RAC Public Review: 7-28-99

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**9907-331 (Withdrawn-replaced by protocol # 0004-393) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer/Antisense/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid DNA/Electroporation/TGF- $\beta$ /Subcutaneous Injection**

Gutheil, John C. and Fakhrai, Habib; Sharp HealthCare, Sidney Kimmel Cancer Center, San Diego, California; *Phase II Study of Antisense TGF- $\beta$  +/- IL-2 Gene Transfected Allogeneic Tumor Cells as a Vaccine in Patients with Stage IIIB and IV Non-Small Cell Lung Cancer.* Sponsor: NovaRx Corporation.

NIH/ORDA Receipt Date: 7-8-99.

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**9907-332 (Open) Gene Therapy/Phase I-II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Interleukin-12 cDNA/Intratumoral Injection**

Colevas, Alexander Dimitrios; Dana-Farber Cancer Institute, Harvard Medical School, Boston, Massachusetts; *A Multi-Center, Open-Label, Multiple Administration, Rising Dose Study of the Safety, Tolerability, and Efficacy of IL-12 Gene Medicine in Patients with Unresectable or Recurrent/Refractory Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: Valentis, Inc.

NIH/ORDA Receipt Date: 7-16-99. Not Selected for RAC Public Review: 8-5-99

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**9908-333 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD34+ Hematopoietic Stem Cells/Retrovirus/Transdominant Rev/Antisense Pol 1/Intravenous Infusion**

Swindells, Susan; University of Nebraska Medical Center, Omaha, Nebraska; Scadden, David; Massachusetts General Hospital, Boston, Massachusetts; Holodniy, Mark; Veterans Affairs Palo Alto Health Care System, Palo Alto, California; and MacGregor, Rob Roy; University of Pennsylvania Hospitals, Philadelphia, Pennsylvania; *A Multicenter Evaluation of the Safety and Efficacy of Hematopoietic Stem Cells Transduced with RevM10polAS (RevM10polAS HSCIP) as Therapy for HIV-1 Infected Persons.* Sponsor: Systemix, Inc.

NIH/ORDA Receipt Date: 8-16-99. Not Selected for RAC Public Review: 9-3-99

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**9908-334 (Under Review) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine cDNA/Ganciclovir/Intraperitoneal Injection**

Alvarez, Ronald D.; Barnes, Mack N.; and Curiel, David T.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Study of FGF2-Fab' Modified Adenovirus Vector Mediated Intraperitoneal Delivery of Herpes Simplex Virus Thymidine Kinase (HSV-TK) Gene and Intravenous Ganciclovir in Previously Treated Ovarian and Extraovarian Patients.*

NIH/ORDA Receipt Date: 8-17-99. Review at a RAC meeting pending; investigators have requested postponement of public review.

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**9908-335 (Open) Gene Therapy/Phase I/Immunotherapy/Cancer/Ovarian/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous or Intradermal Injection**

Dranoff, Glenn; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Study of Vaccination with Lethally Irradiated, Autologous Ovarian Carcinoma Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor*

NIH/ORDA Receipt Date: 8-18-99. Not Selected for RAC Public Review: 9-8-99

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**9908-336 (Open) Gene Marking/Leukemia/In Vitro/CD 34+ Autologous Cord Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous**

Croop, James and Cornetta, Kenneth.; Indiana University School of Medicine; and Kelly, Patrick; Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio; *Post-Transplant Infusion of Fibronectin-Assisted, Retroviral-Mediated Gene-Marked and Ex Vivo Expanded CD34+ Placental and Umbilical Cord Blood Cells*

NIH/ORDA Receipt Date: 8-19-99. Not Selected for RAC Public Review: 9-9-99

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**9908-337 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Severe Combined Immune Deficiency due to adenosine Deaminase Deficiency/In Vitro/Autologous CD34+ Cells from Cord Blood or Bone Marrow/Retrovirus/Adenosine Deaminase cDNA/Intravenous Infusion**

Kohn, Donald B.; Childrens Hospital Los Angeles, University of Southern California, Los Angeles, California; and Brochstein, Joel; Hackensack University Medical Center, Hackensack, New Jersey; *Transduction of CD34+ Cells from the Umbilical Cord Blood of Infants or the Bone Marrow of Children with Adenosine Deaminase (ADA)-Deficient Severe Combined Immunodeficiency (SCID)*

NIH/ORDA Receipt Date: 8-26-99. Publicly Reviewed at the March 2000 RAC meeting

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**9909-338 (Open) Gene Therapy/Phase I/Cancer/Prostate/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p 16 cDNA/Intratumoral Injection**

Gingrich, Jeffrey R.; University of Tennessee, Memphis, Tennessee; *A Tolerance and Efficacy Study of Neoadjuvant Intraprostatic GTx-001 Followed by Radical Prostatectomy in Patients with Locally Advanced Prostate Cancer*. Sponsor: Genotherapeutics, Inc.

NIH/ORDA Receipt Date: 9-2-99. Not Selected for RAC Public Review: 9-29-99

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**9909-339 (Open) Gene Therapy/Phase I-II/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Retrovirus/BRCA1 Gene/Intraperitoneal Administration**

Holt, Jeffrey T.; Vanderbilt University, Nashville, Tennessee, and Tait, David L.; East Carolina University, Greenville, North Carolina; *Ovarian Cancer Gene Therapy with BRCA1*.

NIH/ORDA Receipt Date: 9-13-99. Not Selected for RAC Public Review: 10-1-99

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**9909-340 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD34+ Hematopoietic Stem Cells/Retrovirus/Transdominant Rev/Antisense Pol 1/Intravenous Infusion**

Carabasi, Mathew H.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I/II Study to Evaluate the Safety and Effectiveness of RevM10polAS HSCIP in Late-Stage AIDS Patients Given Intensive Myelosuppressive Conditioning*. Sponsor: Systemix Inc.

NIH/ORDA Receipt Date: 9-17-99. Not Selected for RAC Public Review: 10-7-99

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**9909-341 (Submission Not Complete) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD34+ Cells/Retrovirus/Antisense TAT/Transdominant Rev cDNA/Intravenous**

Tisdale, John; National Institutes of Health, Bethesda, Maryland; *Low Intensity Non-Myeloablative Preparative Conditioning Followed by Transplantation of Genetically Modified HLA-Matched Peripheral Blood Hematopoietic Precursor Cells (PBPC) for Hematologic Malignancies in HIV Positive Adults*

NIH/ORDA Receipt Date: 9-20-99.

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**9910-342 (Open) Gene Therapy/Phase I/Other/Ulcer/In Vivo/Adenovirus/Serotype 5/Platelet Derived Growth Factor (PDGF) cDNA/Intra-Ulcer Injection**

Margolis, David J.; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *Phase I Trial tot Evaluate the Safety of H5.020CMVPDGF-B for the Treatment of a Diabetic Insensate Foot Ulcer*. Sponsor: Institute for Human Gene Therapy, University of Pennsylvania

NIH/ORDA Receipt Date: 10-1-99. Publicly Reviewed at the December 1999 RAC meeting.

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**9910-343 (Open) Gene Therapy/Phase I/Other/Ulcer/In Vivo/Adenovirus/Serotype 5/Platelet Derived Growth Factor (PDGF) cDNA/Intra-Ulcer Injection**

Margolis, David J.; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *Phase I Trial to Evaluate the Safety of H5.020CMVPDGF-B and Limb Compression Bandage for the Treatment of Venous Leg Ulcer (Trial A)*. Sponsor: Institute for Human Gene Therapy, University of Pennsylvania

NIH/ORDA Receipt Date: 10-1-99. Publicly Reviewed at the December 1999 RAC meeting.

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**9910-344 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Adenovirus Type 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intratumoral Injection**

Terris, Martha K.; Palo Alto Veterans Administration Medical Center, Stanford University, Palo Alto, California; *A Phase I/II Dose Finding Trial of the Intraprostatic Injection of Calydon CV787, a Prostate-Specific Antigen Cytolytic Adenovirus, in Patients with Locally Recurrent Prostate Cancer Following Definitive Radiotherapy.* Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 10-13-99. Not Selected for RAC Public Review: 11-2-99

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**9910-345 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Metastatic Prostate Cancer/Vector-Directed Cell Lysis/In Vivo/Adenovirus Type 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate-Specific Antigen/Intravenous Injection**

Wilding, George; University of Wisconsin Comprehensive Cancer Center, Madison, Wisconsin; *A Phase I/II Dose Finding Trial of the Intravenous Injection of Calydon CV787, a Prostate-Specific Antigen Cytolytic Adenovirus, in Patients with Hormone Refractory Metastatic Prostate Cancer.* Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 10-13-99. Publicly Reviewed at the March 2000 RAC meeting

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**9910-346 (Closed) Gene Therapy/Phase II/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration**

Stewart, Duncan J.; St. Michael's Hospital, University of Toronto, Toronto, Canada; Buller, Christopher; University of British Columbia, Vancouver, British Columbia, Canada; Rivard, Alain; University of Montreal, Montreal, Canada; Gregoire, Jean C.; Montreal Heart Institute, Montreal, Canada; Page, Pierre; Hopital du Sacre-Coeur de Montreal, Montreal, Canada; Plante, Sylvain; Laval Hospital, Sainte-Foy, Canada; Archer, Stephen L.; University of Alberta, Alberta, Canada; Sullivan, John; QEII Health Science Center, Halifax, Canada; Dangoisse, Vincent; Hopital Royal Victoria Hospital, Montreal, Canada; Ducas, John; University of Manitoba, Manitoba, Canada; Hilton, J. David; Victoria Heart Institute, Victoria, Canada; Cohen, Eric A. and Bhatnagar, Gopal; Sunnybrook & Women's College Health Sciences Centre, Toronto, Canada; Langlois, Yves; Jewish General Hospital, Montreal, Quebec; Curtis, Michael; Foothills Hospital/University of Calgary, Alberta, Canada; Arnold, J. Malcolm O.; University of Western Ontario, London, Ontario, Canada; Dib, Nabil; Arizona Heart Institute & Foundation; Rajakumar, A. R. J.; Royal University Hospital, Saskatoon, Canada; Frank, Michael; Evanston Northwestern Healthcare, Evanston, Illinois; Lowe, James E.; Duke University Medical Center, Durham, North Carolina; and Mendelsohn, Farrell O.; Cardiology, P.C., Birmingham, Alabama; *A Phase II, Randomized, Multicenter, 26-Week Study to Assess the Efficacy and Safety of CI-1023 Delivered Through Minimally Invasive Surgery Versus Maximum Medical Treatment in Patients with Severe Angina, Advanced Coronary Artery Disease, and No Options for Revascularization.* Sponsor: GenVec, Inc.

NIH/ORDA Receipt Date: 10-12-99. Not Selected for RAC Public Review: 11-5-99  
Study complete: 2-04-03

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**9910-347 (Withdrawn from RAC Review) Gene Therapy/Phase I/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration**

Rosengart, Todd K.; Northwestern Healthcare, Northwestern University, Evanston, Illinois; *Assessment of Direct Administration Via Minimally Invasive Surgery of a Replication Deficient Adenovirus Vector (Ad<sub>CU</sub>VEGF.1) Containing the VEGF cDNA to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease.* Sponsor: R. Crystal, Institute of Genetic Medicine, The New York Presbyterian Hospital-Weill College of Cornell University

NIH/ORDA Receipt Date: 10-14-99. Withdrawn from RAC review: 10-16-00

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**9910-348 (Withdrawn from RAC Review) Gene Therapy/Phase I/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration**

Crystal, Ronald G.; Institute of Genetic Medicine, The New York Presbyterian Hospital-Weill College of Cornell University, New York, New York; *Assessment of Direct Administration Via Minimally Invasive Surgery of a Replication Deficient Adenovirus Vector (Ad<sub>CU</sub>VEGF.1) Containing the VEGF cDNA to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease.*

NIH/ORDA Receipt Date: 10-14-99. Withdrawn from RAC review: 10-16-00

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**9910-349 (Withdrawn-replaced by protocol # 0010-427) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Sweat Duct Epithelium/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intradermal Administration**

Crystal, Ronald G.; Institute of Genetic Medicine, The New York Presbyterian Hospital-Weill College of Cornell University, New York, New York; *Effect of Ad<sub>GV</sub>CFTR.10 on the Cystic Fibrosis Phenotype.*

NIH/ORDA Receipt Date: 10-14-99.

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**9910-350 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Oncogene Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal Administration**

Alberts, David S.; Arizona Cancer Center, University of Arizona, Tucson, Arizona; Wolf, Judith K.; University of Texas, M.D. Anderson Cancer Center, Houston, Texas; and Muntz, Howard; Virginia Mason Medical Center, Seattle, Washington; *A Phase I Dose Escalation Study of Intraperitoneal E1A-Lipid Complex (1:3) with Combination Chemotherapy in Women with Epithelial Ovarian Cancer*. Sponsor: Targeted Genetics Corporation

NIH/ORDA Receipt Date: 10-14-99. Not Selected for RAC Public Review: 11-3-99

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**9910-351 (Open) Gene Therapy/Phase II/Cancer/Angioendothelioma/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Human Interferon- $\alpha$  cDNA/Intratumoral Injection**

Baker, Laurence H.; University of Michigan Medical School, Ann Arbor, Michigan; *An Open-Label, Multiple Administration, Study of the Safety, Tolerability, and Efficacy of IFN- $\alpha$  Gene Medicine in Patients with Malignant Angioendothelioma*. Sponsor: Valentis, Inc.

NIH/ORDA Receipt Date: 10-19-99. Not Selected for RAC Public Review: 11-8-99

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**9910-352 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1102/Leuvectin/Interleukin-2 cDNA/Intratumoral Injection**

Belldegrun, Arie; University of California, Los Angeles Medical Center, Los Angeles, California; Klein, Eric A.; Cleveland Clinic Foundation, Cleveland, Ohio; Corman, John; Virginia Mason Medical Center, Seattle, Washington and Moul, Judd; Walter Reed Army Medical Center, Washington, DC; *Phase I/II Study Evaluating the Safety and Efficacy of Leuvectin Immunotherapy for the Treatment of Locally Recurrent Prostate Cancer Following Radiation Therapy*. Sponsor: Vical Inc.

NIH/ORDA Receipt Date: 10-25-99. Not Selected for RAC Public Review: 11-12-99  
Closed: 6-27-03; follow-up is continuing

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**9911-353 (Submission Not Complete) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/VEGF2-PAD-CL-009/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection**

Annex, Brian H.; Durham VA Medical Center, Durham, North Carolina; *An Open Label Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with Critical Limb Ischemia*. Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 11-5-99.  
Closed: 2-11-00

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**9911-354 (Closed) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/VEGF2-CAD-CL-005/Vascular Endothelial Growth Factor (VEGF) cDNA/Percutaneous Cardiac Catheterization**

Isner, Jeffrey M.; St. Elizabeth's Medical Center, Boston, Massachusetts; *A Placebo-Controlled, Dose-Escalating Study of Intramyocardial Vascular Endothelial Growth Factor 2 (VEGF2) Gene Therapy Administered Using Percutaneous Cardiac Catheterization in Patients with Class III or IV Angina*. Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 11-5-99. Not Selected for RAC Public Review: 1-28-00  
Follow-up has been completed: 11-29-01

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**9911-355 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Anaplastic Astrocytoma/Immunotherapy/In Vitro/Allogeneic Fibroblasts/Lethally Irradiated/Plasmid DNA-Electroporation/IR850-170/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal Injection**

Black, Keith L.; Cedars-Sinai Medical Center, Los Angeles, California; *A Phase I, Open Label, Safety Study of Allogeneic Glioblastoma Tumor Cell Lines (IR850) Mixed with Allogeneic Fibroblasts Genetically Modified to Secrete GM-CSF (IR851) in Patients with Glioblastoma Multiforme or Anaplastic Astrocytoma*. Sponsor: The Immune Response Corporation

NIH/ORDA Receipt Date: 11-12-99. Not Selected for RAC Public Review: 2-3-00

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**9911-356 (Closed) Gene Therapy/Phase I/Cancer/MUC-1 Expressing Tumors/Immunotherapy/In Vivo/Vaccinia Virus/TG4010.01/MUC-1/Interleukin-2/Intramuscular Injection**

Figlin, Robert and Belldegrun, Arie; University of California, Los Angeles Medical Center, Los Angeles, California; *Phase I Bridging Trial of TG4010 as Antigen-Specific Immunotherapy in Patients with MUC-1 Positive Advanced Cancer*. Sponsor: Transgene, Inc.

NIH/ORDA Receipt Date: 11-16-99. Not Selected for RAC Public Review: 12-6-99  
Completed: 9-11-00

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**9911-357 (Closed) Gene Therapy/Phase I-II/Cancer Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1102/Leuvectin/Interleukin-2 cDNA/Intratumoral Injection**

Galanis, Evanthia; Mayo Clinic, Rochester, Minnesota; and Hawkins, Michael; Washington Hospital Center, Washington Cancer Institute, Washington, D.C.; *Protocol for Retreatment with Leuvectin Immunotherapy for Cancer*. Sponsor: Vical Inc.

NIH/ORDA Receipt Date: 11-18-99. Not Selected for RAC Public Review: 12-8-99  
Closed: 6-27-03

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**9911-358 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Liver/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection**

Sung, Max W. and Woo, Savio L. C.; Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenoviral Vector Delivery of the Human Interleukin-12 cDNA by Intratumoral Injection in Patients with Metastatic Breast Cancer to the Liver*.

NIH/ORDA Receipt Date: 11-22-99. Publicly Reviewed at the March 2000 RAC meeting

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**9911-359 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Liver/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection**

Sung, Max W. and Woo, Savio L. C.; Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenoviral Vector Delivery of the Human Interleukin-12 cDNA by Intratumoral Injection in Patients with Primary or Metastatic Colorectal Cancer to the Liver*.

NIH/ORDA Receipt Date: 11-22-99. Publicly Reviewed at the March 2000 RAC meeting

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**9912-360 (Open) Gene Marking/Cancer/Melanoma/In Vitro/Syngeneic Peripheral Blood Lymphocytes/Retrovirus/Neomycin Phosphotransferase Gene/Intravenous Infusion**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma using Cloned Lymphocytes following the Administration of a Nonmyeloablative but Lymphocyte Depleting Regimen*.

NIH/ORDA Receipt Date: 11-22-99. Not Selected for RAC Public Review: 12-31-99

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**9912-361 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/B7 (CD80), HLA-A1 or A2 cDNAs/Subcutaneous Injection**

Podack, Eckhard R.; Cassileth, Peter A.; Sridhar, Kasi; and Savaraj, Niramol; University of Miami, Miami, Florida; *Elicitation of a Cellular Immune Response in Patients with Non-Small Cell Lung Cancer by Immunogenic Tumor Cell Vaccination - A Phase I Study*.

NIH/ORDA Receipt Date: 12-1-99. Not Selected for RAC Public Review: 12-21-99

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**9912-362 (Open) Gene Marking/Cancer/Melanoma/In Vitro/Syngeneic Peripheral Blood Lymphocytes/Retrovirus/Neomycin Phosphotransferase Gene/Intravenous Infusion**

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma Using Cloned Peripheral Blood Lymphocytes Sensitized In Vitro to the gp209-2M Immunodominant Peptide*

NIH/ORDA Receipt Date: 12-16-99. Not Selected for RAC Public Review: 1-7-00

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**9912-363 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Penile Carcinoma/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/adenovirus/Serotype 5/E. coli Cytosine deaminase Gene/Herpes Simplex Thymidine Kinase cDNA/Valacyclovir/Intratumoral Injection**

Miles, Brian J.; Ayala, Gustavo; and Aguilar-Cordova, Estuardo; Baylor College of Medicine, Texas Children's Hospital, Houston, Texas; *Phase I Study of the Replication-Competent, E1B-Attenuated Adenovirus with a CD/HSV-1 TK Fusion Gene and the Oral Administration of Valacyclovir in Adults with Penile Cancer*.

NIH/ORDA Receipt Date: 12-20-99. Publicly Reviewed at the March 2000 RAC meeting

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**9911-364 (Open) Gene Therapy/Phase I-II/Infectious Disease/Epstein-Barr Virus (EBV) and Cytomegalovirus Diseases/In Vitro/EBV and CMV-Specific Cytotoxic T Lymphocytes/Retrovirus/Cytomegalovirus pp65 Gene/Intravenous**

Lucas, Kenneth G.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I-II Trial to Examine the Toxicity of CMV and EBV Specific Cytotoxic T Lymphocytes When Used for Prophylaxis Against EBV and CMV Disease in Recipients of CD34+ Selected/T Cell Depleted Stem Cell Transplants.*

NIH/ORDA Receipt Date: 11-26-99. Not Selected for RAC Public Review: 1-3-00

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**9912-365 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD4+ T Cells/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous Infusion**

Aronson, Naomi; Walter Reed Army Medical Center, Washington, D.C.; *A Phase I/II Study of the Safety, Survival, and Trafficking of Autologous CD4-zeta Gene-Modified T Cells With and Without Exogenous Interleukin-2 in HIV-Infected Patients.* Sponsors: University of Pennsylvania and Cell Genesys, Inc.

NIH/ORDA Receipt Date: 12-22-99. Not Selected for RAC Public Review: 4-14-00

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**9912-366 (Open) Gene Therapy/Phase III/Cancer/Squamous Cell Carcinoma of the Head and Neck (SCCHN)/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5 p53 cDNA/Intratatumoral Injections**

Hamm, John T.; University of Louisville, Norton Healthcare, Louisville, Kentucky; Haigentz, Missak; Montefiore Medical Center, Bronx, New York; Arquette, Mathew; Washington University School of Medicine, Barnard Cancer Center, St. Louis, Missouri; Cullen, Kevin J.; Georgetown University Medical Center, Washington, D.C.; Goodwin, W. Jarrard; University of Miami, Miami, Florida; Flood, William A.; The Milton S. Hershey Medical Center, Hershey, Pennsylvania; Yoo, George; University Health Center, Detroit, Michigan; Kreml, Greg; The University of Oklahoma, Oklahoma City, Oklahoma; Turpeenniemi-Hujanen, Taina; OULU University Hospital, Oulu, Finland; Kellokumpu-Lehtinen, Pirkko; Tampere University Hospital, Tampere, Finland; Brockstein, Bruce; Evanston Northwestern Healthcare, Evanston, Illinois; Cobb, Patrick; Billings Oncology Associates, Billings, Montana; Williamson, Stephen; University of Kansas Medical Center, Kansas City, Kansas; Burke, Brian; The Vanderbilt Clinic/Vanderbilt University Medical Center, Nashville, Tennessee; Carrato Mena, Alfredo; Hospital General De Elche, Elche (Spain); Barnadas, Agusti; Hospital Universitari Germans Trias i Pujol, Barcelona, Spain; Trigo, J. Ma.; Hospital General Vall d'Hebron, Barcelona, Spain; Cortes-Funes, Hernan; Hospital 12 de Octubre, Madrid, Spain; Constenla, Manuel; Complejo Hospitalario De Pontevedra, Pontevedra, Spain; Sanchez, Emilio Fonseca; Hospital Clinico de Salamanca, Salamanca, Spain; Ruiperez, Andres Cervantes; Hospital Clinico Universitario, Valencia, Spain; Guillem, Vicente; Instituto Valenciano de Oncologia, Valencia, Spain; Nathan, Cherie-Ann; Louisiana State University, Shreveport, Louisiana; Agarwala, Sanjiv; University of Pittsburgh, Pittsburgh, Pennsylvania; Rosen, Fred; The University of Illinois at Chicago, Chicago, Illinois; Breau, Randall; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Giguere, Jeffrey; Cancer Center of the Carolinas, Greenville, South Carolina; Trask, Douglas; University of Iowa Hospitals and Clinics, Iowa City, Iowa; Zitsch, Robert; University of Missouri Health Care, Columbia, Missouri; Hrushesky, William; J. M., Dorn Veterans Affairs Medical Center, Columbia, South Carolina; Clayman, Gary; University of Texas, M.D. Anderson Cancer Center, Houston, Texas; Guthrie, Troy H., Jr.; University of Florida, Jacksonville, Florida; Slolomon, William; SUNY Health Science Center at Brooklyn, Brooklyn, New York; Law, Amy; Geisinger Medical Center, Danville, Pennsylvania; Trask, Douglas; University of Iowa Health Care, Iowa City, Iowa; Nemecek, Andrew; Tulane University School of Medicine, New Orleans, Louisiana; Villaret, Douglas; University of Florida, Gainesville, Florida; Van Echo, David; University of Maryland School of Medicine, Baltimore, Maryland; McCaffrey, Thomas V.; H. Lee Moffitt Cancer Center and Research Institute, Tampa, Florida; Wheeler, Richard H.; Huntsman Cancer Institute, Salt Lake City, Utah; Chen, Amy; Emory University, Atlanta, Georgia; Gal, Thomas, Jr.; University of Washington Medical Center, Seattle, Washington; Axelrod, Rita; Thomas Jefferson University, Philadelphia, Pennsylvania; Kane, Madeleine; Denver Veteran's Administration Medical Center, Denver, Colorado; Levine, Marshall; Greater Baltimore Medical Center, Baltimore, Maryland; Weisman, Robert; University of California, San Diego, San Diego, California; and Bier-Laning, Carol M.; Edward Hines, Jr. VA Hospital, Hines, Illinois; *A Phase III Multi-Center, Open-Label, Randomized Study to Compare the Overall Survival and Safety of Bi-Weekly Intratumoral Administration of INGN 201 Versus Weekly Methotrexate in 240 Patients with Refractory Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: Aventis Pharmaceuticals - Gencell Division (formerly Rhone-Poulenc Rorer)

NIH/ORDA Receipt Date: 12-28-99. Publicly Reviewed at the March 2000 RAC meeting

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**9912-367 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous**

Vieweg, Johannes; Duke University Medical Center, Durham, North Carolina; *Active Immunotherapy of Metastatic Renal Cell Carcinoma Using Autologous Dendritic Cells Transfected with Autologous Renal Tumor RNA.*

NIH/ORDA Receipt Date: 12-28-99. Not Selected for RAC Public Review: 1-14-00  
Closed to enrollment: 2-13-03

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**9912-368 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen/B7.1 (CD80)/Intramuscular or Intradermal Injection**

Dahut, Bill; National Naval Medical Center, Bethesda, Maryland; and Gulley, James; National Institutes of Health, Bethesda, Maryland; *A Randomized Phase II Study of a PSA-Based Vaccine in Patients with Localized Prostate Cancer Receiving Standard Radiotherapy.*

NIH/ORDA Receipt Date: 12-29-99. Not Selected for RAC Public Review: 3-7-00

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**0001-369 (Open) Gene Therapy/Phase I/Immunotherapy/Cancer/Myelodysplasia or Acute Myelogenous Leukemia (AML)/In Vitro/Autologous Acute Myeloblastic Leukemia Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous or Intradermal Injection**

DeAngelo, Daniel J.; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Study of Vaccination with Lethally Irradiated, Autologous Acute Myeloblastic Leukemia Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor in Patients with Advanced Myelodysplasia or Acute Myelogenous Leukemia.*

NIH/OBA Receipt Date: 1-3-00. Not Selected for RAC Public Review: 1-24-00

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**0001-370 (Open) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Fanconi Anemia Complementation Group A and C cDNA/Intravenous**

Croop, James M.; Indiana University School of Medicine, Indianapolis, Indiana; *Gene Therapy for Patients with Fanconi Anemia: A Pilot Study.*

NIH/OBA Receipt Date: 1-6-00. Not Selected for RAC Public Review: 2-4-00

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**0001-371 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Hemophilia B/In Vivo/Adeno-Associated Virus/Factor IX Gene/Intrahepatic Artery Administration**

Glader, Bertil; Stanford University, Stanford, California; and Manno, Catherine; The Children's Hospital of Philadelphia, Philadelphia, Pennsylvania; *A Phase I Safety Study in Patients with Severe Hemophilia B (Factor IX Deficiency) Using Adeno-Associated Viral Vector to Deliver the Gene for Human Factor IX into the Liver.*

NIH/OBA Receipt Date: 1-7-00. Publicly Reviewed at the March 2000 RAC meeting

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**0001-372 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Hemophilia A/In Vivo/Helper-Dependent (Gutted) Adenovirus/Factor VIII cDNA/Intravenous Injection**

White II, Gilbert; University of North Carolina School of Medicine, Chapel Hill, North Carolina; Thompson, Arthur; University of Washington, Seattle, Washington; and Gruppo, Ralph A.; Children's Hospital Medical Center, Cincinnati, Ohio; *A Phase 1, Single-Dose, Dose-Escalation Study of MiniAdFVIII Vector in Patients with Severe Hemophilia A.* Sponsor: Coraetus Genetics, Inc. (formerly GenStar Therapeutics Corporation)

NIH/OBA Receipt Date: 1-12-00. Publicly Reviewed at the September 2000 RAC meeting  
Closed: 2-13-03

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**0001-373 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen/B7.1 (CD80)/Intramuscular or Intradermal Injection**

Arlen, Philip M.; National Naval Medical Center and National Institutes of Health, Bethesda, Maryland; *A Randomized Phase II Study of Either Immunotherapy with a Regimen of Recombinant Pox Viruses that Express PSA/B7.1 Plus Adjuvant GM-CSF and IL-2 or Hormone Therapy with Nilutamide in Patients with Hormone Refractory Prostate Cancer and No Radiographic Evidence of Disease.*

NIH/OBA Receipt Date: 1-10-00. Not Selected for RAC Public Review: 3-7-00

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**0001-374 (Withdrawn-replaced by 0007-407) Gene Therapy/Phase I/Coronary Artery Disease/In Vivo/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1 $\alpha$ /VP16 cDNA/Cardiac Administration**

*A Phase I Open Label, Escalating Dose, Multi-Center Study of Ad2/Hypoxia Inducible Factor (HIF)-1 $\alpha$ /VP16 Gene Transfer Administered by Intramyocardial Injection During Coronary Artery Bypass Grafting (CABG) Surgery in Patients with Areas of Viable and Underperfused Myocardium not Amenable to Bypass Grafting or Percutaneous Intervention and the related follow-up study A Phase I Open Label, Multi-Center Extension Study of Ad2/Hypoxia Inducible Factor (HIF)-1 $\alpha$ /VP16 Gene Transfer Administered by Intramyocardial Injection During Coronary Artery Bypass Grafting (CABG) Surgery in Patients with Areas of Viable and Underperfused Myocardium not Amenable to Bypass Grafting or Percutaneous Intervention.* Sponsor: Genzyme Corporation.

NIH/OBA Receipt Date: 1-13-00.

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**0001-375 (Withdrawn-replaced by protocol # 0010-425) Gene Therapy/Phase I/Other Disorders/Hip Fracture/In Vivo/Plasmid DNA/Collagen Sponge/Parathyroid Hormone cDNA/Bone Administration**

*A Phase I Safety, Tolerance and Pharmacokinetic Study of Mat-100 in Elderly Patients with Fresh Fracture of the Hip.* Sponsor: Selective Genetics, Inc.

NIH/OBA Receipt Date: 1-13-00.

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**0001-376 (Open) Gene Therapy/Phase I/Cancer/Non-Hodgkin's Lymphoma/Chemoprotection/Fusion Gene of a Mutant Dihydrofolate Reductase and Cytidine Deaminase/In Vitro/Autologous Peripheral Blood CD34+ Cells/Retrovirus/Intravenous Infusion**

Bertino, Joseph; Memorial Sloan Kettering Cancer Center, New York, New York; *A Gene Therapy Based Myeloprotection Strategy Using a Mutant Dihydrofolate Reductase - Cytidine Deaminase Fusion Gene for the Treatment of Refractory or Relapsed Non-Hodgkin's Lymphoma.*

NIH/OBA Receipt Date: 1-13-00. Not Selected for RAC Public Review: 2-3-00

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**0001-377 (Withdrawn from RAC Review) Gene Therapy/Phase I/Monogenic Disease/Fabry Disease/In Vitro/Autologous Mesenchymal Stem Cells/Retrovirus/ $\alpha$ -Galactosidase A cDNA/Immunoisolation Device/Subcutaneous Implantation**

Medin, Jeffrey A.; University of Illinois at Chicago, Chicago, Illinois; *A Phase I Trial of Retroviral Transduction of Autologous Mesenchymal Stem Cells from Patients with Fabry Disease with Alpha-Galactosidase A cDNA and Implantation Via an Immunoisolation Device.* Sponsor: Osiris Therapeutics, Inc.

NIH/OBA Receipt Date: 1-13-00. Withdrawn from RAC review: 3-1-00

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**0002-378 (Open) Gene Therapy/Phase II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Interferon- $\alpha$ /Interleukin-12 cDNA/Intratumoral Injection**

McQuone, Shelly J.; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Multicenter, Open-Label, Multiple Administration, Study of the Safety, Tolerability and Efficacy of IFN $\alpha$ /IL-12 Combination Gene Therapy in Patients with Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 2-9-00. Not Selected for RAC Public Review: 8-8-00

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**0001-379 (Submission Not Complete) Gene Therapy/Phase I/Immunotherapy/Cancer/Colon/Adenovirus/Serotype 5/GA733-2 Antigen cDNA/Intradermal Injection**

Eck, Stephen L.; University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *Phase I Trial of Intradermal Adenovirus GA733 Vaccine for Advanced Colorectal Cancer.*

NIH/OBA Receipt Date: 1-13-00.

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**0001-380 (Under Review) Gene Therapy/Phase I/Monogenic Disease/Amyotrophic Lateral Sclerosis/In Vivo/Adeno-Associated Virus/Excitatory Amino Acid Transporter 2 (EAAT2) cDNA/Percutaneous Cervical Injection**

During, Matthew J. and Simeone, Frederick A.; Thomas Jefferson University, Philadelphia, Pennsylvania; *Clinical Trial in Amyotrophic Lateral Sclerosis Patients Using Gene Transfer of the EAAT2 Gene in the Cervical Spinal Cord.*

NIH/OBA Receipt Date: 1-13-00. Review at a RAC meeting pending; investigators have requested postponement of public review.

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**0001-381 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Canavan Disease/In Vivo/Adeno-Associated Virus/Aspartoacylase cDNA/Stereotactic Intracranial Administration**

Leone, Paola and Feely, Michael; Cooper Health System, Camden, New Jersey; *Gene Therapy of Canavan Disease using AAV for Brain Gene Transfer.*

NIH/OBA Receipt Date: 1-13-00. Publicly Reviewed at the March 2000 RAC meeting

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**0001-382 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous Neuroblastoma Cells/Lethally Irradiated/Adenovirus/Serotype 5/Interleukin-2 cDNA/Subcutaneous Injection**

Russell, Heidi; Baylor College of Medicine, Houston, Texas; *A Pilot Study of Gene Modified Autologous Neuroblastoma Vaccine for the Post-Chemotherapy Treatment of High Risk Neuroblastoma.*

NIH/OBA Receipt Date: 1-14-00.

Closed to new accrual, follow-up continues: 3-17-04

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**0001-383 (Withdrawn) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Catheterization**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Phase IIb Multicenter, Randomized, Controlled Study of Direct Intramyocardial Injection of pVGL.1 (VEGF2) Versus Maximum Medical Therapy in Patients with Class III or IV Angina.*  
Sponsor: Coraetus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/OBA Receipt Date: 1-18-00.

Withdrawn from consideration, no individuals enrolled: 11-29-01

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**0001-384 (Withdrawn) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Catheterization**

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Double-Blind, Placebo-Controlled, Continuation Study of Intramyocardial pVGL.1 (VEGF2) Administered by Percutaneous Cardiac Catheterization in Patients with Class III or IV Angina.*  
Sponsor: Coraetus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/OBA Receipt Date: 1-18-00.

Withdrawn from consideration, no individuals enrolled: 11-29-01

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**0001-385 (Closed) Gene Therapy/Phase I-II/Immunotherapy/Cancer/Non-Small Cell Lung Carcinoma (NSCLC)/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Subcutaneous Injection**

Smith II, John W.; Providence Portland Medical Center, Portland, Oregon; Jablons, David; University of California, San Francisco, San Francisco, California; and Sterman, Daniel; University of Pennsylvania, Philadelphia, Pennsylvania; *Phase I/II Study of GM-CSF Gene-Modified Autologous Tumor Vaccines in Early and Advanced Stage Non-Small Cell Lung Cancer (NSCLC).* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 1-20-00. Not Selected for RAC Public Review: 2-9-00

Closed to enrollment: 4-21-03

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**0001-386 (Open) Gene Therapy/Phase II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Irradiated/Canarypox Virus/B7.1 (CD80) cDNA/Subcutaneous Injection**

Antonia, Scott J.; H. Lee Moffitt Cancer Center, University of South Florida, Tampa, Florida; *Phase II Study of a B-7.1 Gene Modified Autologous Tumor Cell Vaccine and Systemic IL-2 for Patients with Stage IV Renal Cell Carcinoma.*

NIH/OBA Receipt Date: 1-24-00. Not Selected for RAC Public Review: 2-29-00

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**0001-387 (Closed) Gene Therapy/Phase II/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration**

Epstein, Stephen; Cardiovascular Research Institute, Washington, D.C.; Dib, Nabil; Arizona Heart Institute & Foundation, Phoenix, Arizona; Cohen, Barry M.; Morristown Memorial Hospital, Morristown, New Jersey; and Moses, Jeffrey W.; Lenox Hill Heart Hospital, New York, New York; *A Randomized, Double-Blind, Placebo-Controlled, Multicenter, 12-Week Follow-up, Pilot Study of the Tolerability and Feasibility of Administering AD<sub>GV</sub>VEGF<sub>121.10</sub> (CI-1023) Via the Biosense Intramyocardial Injection Device to Patients with Advanced Coronary Artery Disease.* Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 1-27-00. Not Selected for RAC Public Review: 2-24-00

Closed: 8-18-03

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**0002-388 (Closed) Gene Therapy/Phase II/Other/Peripheral Arterial Disease/In Vivo/Ischemic Lower Limb/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Intramuscular Injection**

Rajagopalan, Sanjay; University of Michigan Medical Center, Ann Arbor, Michigan; Chaikof, Elliot; Emory University School of Medicine, Atlanta, Georgia; Deitcher, Steven; The Cleveland Clinic Foundation, Cleveland, Ohio; Rhee, Robert Y.; University of Pittsburgh, Pittsburgh, Pennsylvania; Corson, John D.; The University of Iowa Hospitals and Clinics, Iowa City, Iowa; Mohler, Emile R.; University of Pennsylvania Health System, Philadelphia, Pennsylvania; Jaff, Michael; Cardiovascular Research Institute, Washington, DC; Lowman, Bruce G.; Watson Clinic Center for Research, Lakeland, Florida; Blebea, John; Penn State College of Medicine, Hershey, Pennsylvania; Hirsch, Alan T.; University of Minnesota, Minneapolis, Minnesota; Annex, Brian H.; Duke University Medical Center, Durham, North Carolina; Guzman, Raul; Vanderbilt University Medical Center, Nashville, Tennessee; Tenaglia, Alan; Tulane University Health Sciences Center, New Orleans, Louisiana; Azrin, Michael; University of Connecticut Health Center, Farmington, Connecticut; Gagne, Paul; New York University School of Medicine, New York, New York; Dib, Nabil; Arizona Heart Institute & Foundation, Phoenix, Arizona; Garza, Luis; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Hermiller, James; The Care Group, Indianapolis, Indiana; Mendelsohn, Farrell; Baptist Health System, Birmingham, Alabama; Miller, Julie M.; Johns Hopkins University, Baltimore, Maryland; Anderson, R. David; Sarasota Memorial Healthcare System, Sarasota, Florida; and Davies, Mark G.; University of Rochester Medical Center, Rochester, New York; *A Double-Blind, Randomized, Placebo-Controlled, Dose-Ranging, 26-Week Study to Assess the Safety and Efficacy of CI-1023 (AD<sub>GV</sub>VEGF<sub>121.10</sub>) in Peripheral Arterial Disease Patients with Severe, Disabling Intermittent Claudication*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 2-2-00. Not Selected for RAC Public Review: 3-27-00  
Closed: 8-18-03

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**0002-389 (Closed) Gene Therapy/Phase I/Cancer/Liver Metastasis of Colorectal Carcinoma/Immunotherapy/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Interleukin-2 cDNA/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection**

Sung, Max W.; Mount Sinai School of Medicine, New York, New York; *Phase I/II Trial of Combination Adenoviral Vector Delivery of the Human Recombinant Interleukin-2 Gene and the Herpes Simplex Virus Thymidine Kinase Gene by Intratumoral Injection and Followed by Intravenous Ganciclovir in Patients with Hepatic Metastases from Colorectal Cancer*.

NIH/OBA Receipt Date: 2-4-00. Not Selected for RAC Public Review: 3-10-00  
No subjects received study agent: May 2000

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**0003-390 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD 34+ Hematopoietic Stem Cells/Retrovirus/Transdominant Rev/Intravenous Infusion**

Kohn, Donald B.; Childrens Hospital Los Angeles, University of Southern California, Los Angeles, California; *Retroviral-Mediated Transfer of the RevM10 and FX Genes into CD 34+ Cells from the Bone Marrow of HIV-1 Infected Children*.

NIH/OBA Receipt Date: 3-1-00. Not Selected for RAC Public Review: 3-21-00

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**0002-391 (Closed) Gene Therapy/Phase II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical-1102/Leuvectin/Interleukin-2 cDNA/Intratumoral Injection/Vical Protocol VCL-1102-204**

Thompson, John A.; University of Washington School of Medicine, Seattle, Washington; Hawkins, Michael; Washington Hospital Center, Washington Cancer Institute, Washington, D.C.; Figlin, Robert A.; University of California Los Angeles Medical Center, Los Angeles, California; Lee, Fa-Chyi; University of New Mexico Cancer Research and Treatment Center and University Hospital, Albuquerque, New Mexico; Ernstoff, Marc S.; Dartmouth Hitchcock Medical Center, Lebanon, New Hampshire; Bukowski, Ronald; The Cleveland Clinic Foundation, Cleveland, Ohio; Morse, Michael A.; Duke University Medical Center, Durham, North Carolina; and Amato, Robert; Baylor College of Medicine, Houston, Texas; *Phase II Study of Leuvectin in Patients with Metastatic Renal Cell Carcinoma*. Sponsor: Vical Inc.

NIH/OBA Receipt Date: 2-14-00. Not Selected for RAC Public Review: 3-6-00  
Notification from sponsor that study is closed: 6-29-01.

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**0003-392 (Closed) Gene Therapy/Phase I-II/Cancer/Non-Hodgkin's B-Cell Lymphoma/Mantle Cell Lymphoma/Immunotherapy/In Vivo/Naked Plasmid DNA/Tumor Idiotypic/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intramuscular and Intradermal Injections/Vical Protocol VCL-1642-101**

Levy, Ronald; Stanford University School of Medicine, Stanford, California; *Phase I/II Study of Vaccine Therapy for B-Cell Lymphoma Utilizing Plasmid DNA Coding for Tumor Idiotypic*. Sponsor: Vical Inc.

NIH/OBA Receipt Date: 3-17-00. Not Selected for RAC Public Review: 4-6-00

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**0004-393 (Open) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer/Antisense/In Vitro/Allogeneic Tumor Cells/Irradiated/Plasmid DNA-Electroporation/TGF- $\beta$ /Subcutaneous Injection**

Sobol, Robert and Bodkin, David; Sharp Health Care, Sidney Kimmel Cancer Center, San Diego, California; Batra, Raj K.; University of California, Los Angeles and West Los Angeles Veteran's Administration Medical Center, Los Angeles, California; Dillman, Robert O.; Hoag Cancer Center, Newport Beach, California; Nemunaitis, John J.; Mary Crowley Medical Research Center, Dallas, Texas; and Schwarzenberger, Paul O.; Louisiana State University Medical Center, New Orleans, Louisiana; *Phase II Study of a TGF- $\beta$ 2 Antisense Gene Modified Allogeneic Tumor Cell Vaccine in Patients with Stages II-IV Non-Small Cell Lung Cancer*. Sponsor: NovaRx

NIH/OBA Receipt Date: 4-3-00. Not Selected for RAC Public Review: 5-2-00

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**0005-394 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/Tyrosinase cDNA/Intramuscular Injection**

Wolchok, Jedd; Memorial Sloan-Kettering Cancer Center, New York, New York; *Vaccination of AJCC Stage III and IV Melanoma Patients with Human and Mouse Tyrosinase DNA Vaccines: A Phase I Trial to Assess Safety and Immune Response*.

NIH/OBA Receipt Date: 5-1-00. Not Selected for RAC Public Review: 5-19-00  
Closed to accrual: 5-25-04

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**0005-395 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Type 5/MART-1 Melanoma Antigen/gp100 Melanoma Antigen/Intradermal Injection**

Haluska, Frank G; Harvard Medical School, Boston, Massachusetts; Cunningham, Charles; US Oncology, Dallas, Texas; Ernstoff, Marc; Dartmouth Hitchcock Medical Center, Lebanon, New Hampshire; and Richards, Jon M.; Oncology Specialists, S. C., Chicago, Illinois; *A Phase I/II Trial Investigating the Safety and Immunogenicity of Adenoviruses Encoding the Melan-A/MART-1 and gp100 Melanoma Antigens Administered Intradermally to Patients with Stage II-IV Melanoma*. Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 5-1-00. Not Selected for RAC Public Review: 9-14-00

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**0005-396 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Colon Carcinoma (Hepatic Metastasis)/Herpes Simplex Virus Type 1/Tumor Lysis/Intrahepatic Artery Administration**

Fong, Yuman; Memorial Sloan Kettering Cancer Center, New York, New York; *A Phase I, Open -Label, Dose-Escalating Study of the Safety, Tolerability, and Anti-tumor Activity of a Single Intrahepatic Injection of a Genetically Engineered Herpes Simplex Virus, NV1020, in Subjects with Adenocarcinoma of the Colon with Metastasis to the Liver* and the associated, long-term follow-up protocol: *Long-Term Follow-Up of the Safety and Survival of subjects with Adenocarcinoma of the Colon with Metastasis to the Liver Who Enrolled in a Phase I Dose-Escalating Study Evaluating a Genetically Engineered Herpes Simplex Virus, NV1020*. Sponsor: NeuroVir Therapeutics, Inc.

NIH/OBA Receipt Date: 5-2-00. Publicly Reviewed at the June 2000 RAC meeting  
Enrollment completed: November 2002

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**0005-397 (Closed) Gene Therapy/Phase I/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration (Catheter)**

Sanborn, Timothy A.; Joan and Sanford I. Weill Medical College, Cornell University, New York, New York; *A Feasibility Study of Catheter-Based Administration of a Replication Deficient Adenovirus Vector (Ad<sub>CV</sub>VEGF.1) to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease*. Sponsor: R. Crystal, M.D.

NIH/OBA Receipt Date: 5-3-00. Not Selected for RAC Public Review: 5-23-00  
Closed, never initiated, no individuals enrolled: 8-22-03

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**0005-398 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/Tropism-Modified Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Somatostatin Receptor cDNA/Ganciclovir/Intraperitoneal Injection**

Barnes, Mack N.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Study of a Tropism Modified Adenovirus Vector for Intraperitoneal Delivery of Therapeutic Genes in Ovarian and Extraovarian Cancer Patients*.

NIH/OBA Receipt Date: 5-3-00. Publicly Reviewed at the December 2000 RAC meeting

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**0005-399 (Closed) Gene Therapy/Phase I/Cancer/Solid Tumors/Immunotherapy/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection**

Guha, Chandan and Mani, Sridhar; Albert Einstein College of Medicine, Bronx, New York; Hanna, Nader; University of Kentucky Medical Center, Lexington, Kentucky; Nemunaitis, John; US Oncology, Dallas, Texas; Richards, Donald A.; Tyler Cancer Center, Tyler, Texas; and Rosemurgy, Alexander; University of South Florida, Tampa, Florida; *An Open-Label, Phase I, Dose-Escalation Study of Tumor Necrosis Factor-alpha (TNFerade™ Biologic) Gene Therapy with Radiation Therapy for Locally Advanced, Recurrent, or Metastatic Solid Tumors.* Sponsor: GenVec

NIH/OBA Receipt Date: 5-3-00. Not Selected for RAC Public Review: 5-23-00  
Closed: January 2003

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**0005-400 (Open) Gene Therapy/Phase I/Cancer/Lymphoma/Chemoprotection/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Intravenous Infusion**

Becker, Pamela S.; University of Massachusetts Memorial Health Care, Worcester, Massachusetts; *Transfer of the Multidrug Resistance Gene, MDR-1, to Hematopoietic Progenitors from Patients with High Risk Lymphoma.*

NIH/OBA Receipt Date: 5-3-00. Not Selected for RAC Public Review: 6-5-00

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**0005-401 (Open) Gene Therapy/Phase II/Cancer/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous Leukemic Cells/Adenovirus/Serotype 5/CD154 cDNA/Intravenous Infusion**

Gribben, John; Dana-Farber Cancer Institute, Boston, Massachusetts; and Millard, Frederick; University of California-San Diego Medical Center, San Diego, San Diego, California; *Open-Label, Multicenter, Phase II Study of Autologous Ad-CD154 Expressing Transduced CLL Cells in B Cell Chronic Lymphocytic Leukemia Subjects Enrolled in Two Parallel Arms.* Sponsor: Tragen Pharmaceuticals (formerly Immunogenex, Inc.)

NIH/OBA Receipt Date: 5-3-00. Not Selected for RAC Public Review: 5-23-00

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**0006-402 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Plasmid DNA/Electroporation/CE7R-Specific scFvFc-Zeta T Cell Receptor/Intravenous Infusion**

Jensen, Michael; City of Hope National Medical Center, Duarte, California; *Phase I Study to Evaluate the Safety of Cellular Immunotherapy for Recurrent/Refractory Neuroblastoma Using Genetically-Modified Autologous CD8+ T Cell Clones.*

NIH/OBA Receipt Date: 6-2-00. Not Selected for RAC Public Review: 6-22-00

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**0006-403 (Closed) Gene Therapy/Phase IIb/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration**

Iskandrian, Ami E.; University of Alabama at Birmingham, Birmingham, Alabama; Churchill, David; North West Arkansas Heart and Vascular Center, Fayetteville, Arkansas; Gammon, Roger S.; Austin Heart, P.A., Austin, Texas; Ghali, Jalal K.; Cardiac Centers of Louisiana, LLC, Shreveport, Louisiana; Grines, Cindy L.; William Beaumont Hospital, Royal Oak, Michigan; Helmer, Gregory A.; Minnesota Heart Clinic, P. A., Edina, Minnesota; Kleiman, Neal S.; Baylor College of Medicine, Houston, Texas; Rade, Jeffrey J.; The Johns Hopkins Hospital, Baltimore, Maryland; Rowe, Steven K.; Heartland Health Center, St. Joseph, Missouri; Watkins, Matthew W.; Fletcher Allen Health Care, Burlington, Vermont; and Uretsky, Barry; University of Texas, Galveston, Galveston, Texas; *A Randomized, Double-Blind, Placebo Controlled Study to Evaluate the Effect of Ad5FGF-4 on Myocardial Perfusion Defect Size and Safety in Patients with Stable Angina.* Sponsor: Berlex Laboratories

NIH/OBA Receipt Date: 6-5-00. Not Selected for RAC Public Review: 8-18-00  
Completed: 09-03

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**0006-404 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase II/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) cDNA/Aerosol Administration**

Moss, Richard B.; Stanford University School of Medicine, Palo Alto, California; Waltz, David; Children's Hospital, Boston, Massachusetts; Rodman, David; University of Colorado Health Sciences Center, Denver, Colorado; Spencer, L. Terry; Virella-Lowell, Isabel; Brantly, Mark; and Flotte, Terry; University of Florida, Gainesville, Florida; Zeitlin, Pamela; Johns Hopkins University, Baltimore, Maryland; Aitken, Moira; University of Washington, Seattle, Washington; Milla, Carlos; University of Minnesota, Minneapolis, Minnesota; and Clancy, John Paul; University of Alabama at Birmingham, Birmingham, Alabama; *A Multicenter, Double-Blind, Placebo-Controlled, Phase II Study of Aerosolized AAVCF in Cystic Fibrosis Patients with Mild Lung Disease.* Sponsor: Targeted Genetics

NIH/OBA Receipt Date: 6-12-00. Publicly Reviewed at the September 2000 RAC meeting  
Enrollment is complete: 10-3-02

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**0006-405 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD 80)/ICAM-1/LFA-3/Intramuscular Or Intradermal Injection**

Marshall, John L.; Georgetown University Medical Center, Washington, D.C.; *A Phase I Study of Sequential Vaccinations with Fowlpox-CEA(6D)-TRICOM (B7.1/ICAM-1/LFA-3) Alone, OR in Combination with Vaccinia-CEA(6D)-TRICOM, and the Role of GM-GSF, in Patients with CEA Expressing Carcinomas.*

NIH/OBA Receipt Date: 6-12-00. Not Selected for RAC Public Review: 11-7-00

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**0006-406 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Peripheral Artery Disease/Anemia of End Stage Renal Disease (ESRD)/In Vitro/Autologous Vascular Smooth Muscle Cells/Retrovirus/Erythropoietin (EPO) cDNA/Vascular Grafts Lined with Transduced Smooth Muscle Cells**

Muczynski, Kimberly A. and Osborne, William R. A.; University of Washington School of Medicine, Seattle, Washington; *Erythropoietin Administration in Hemodialysis Patients Using Vascular Grafts Lined with Transduced Smooth Muscle Cells.*

NIH/OBA Receipt Date: 6-13-00. Publicly Reviewed at the September 2000 RAC meeting

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**0007-407 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Coronary Artery Disease/In Vivo/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1 $\alpha$ /VP16 cDNA/Cardiac Administration/CAD-HIF-004-99**

Rosengart, Todd K; Northwestern University Medical School, Evanston, Illinois; McCurry, Kenneth; University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; Cmolik, Brian L.; University Hospitals of Cleveland, Cleveland, Ohio; Landolfo, Kevin P.; Duke University Medical Center, Durham, North Carolina; Dullum, Mercedes; Washington Hospital Center, Washington, DC; Lattouf, Omar; Emory University School of Medicine, Atlanta, Georgia; Fontana, Gregory; Cedars-Sinai Medical Center, Beverly Hills, California; Chronos, Nicolas; Atlanta Cardiology Research Institute, Atlanta, Georgia; Henry, Timothy; Minneapolis Heart Institute Foundation, Minneapolis, Minnesota; and DiScipio, Anthony; Dartmouth-Hitchcock Medical Center Hanover, New Hampshire; *A Phase I, Double-blind, Placebo Controlled, Escalating Dose, Multi-center Study of Ad2/Hypoxia Inducible Factor (HIF)-1 $\alpha$ /VP16 Gene Transfer Administration by Intramyocardial Injection During Coronary Artery Bypass Grafting (CABG) Surgery in Patients with Areas of Viable and Underperfused Myocardium not Amenable to Bypass Grafting or Percutaneous Intervention.* Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 7-31-00. Publicly Reviewed at the September 2000 RAC meeting

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**0007-408 (Open) Gene Therapy/Phase I-II/Cancer/B-Cell Chronic Lymphocytic Leukemia/Immunotherapy/In Vivo/Naked Plasmid DNA/Tumor Idiotype/Intramuscular Injection**

Garcia-Manero, Guillermo; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I/II Study of Idiotypic Vaccination for Chronic Lymphocytic Leukemia using a Genetic Approach.*

NIH/OBA Receipt Date: 7-31-00. Not Selected for RAC Public Review: 8-18-00

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**0007-409 (Open) Gene Therapy/Phase I/Cancer/Lung Cancer/Immunotherapy/In Vivo/Cationic Liposome Complex/Interleukin-2 cDNA/Intravenous Injection**

Hainsworth, John Daniel; Sarah Cannon Cancer Center, Centennial Medical Center, Nashville, Tennessee; and Antonia, Scott; H. Lee Moffitt Cancer Center, Tampa, Florida; *A Phase I, Multi-Center, Open-Label, Dose-Escalation Study of the Safety and Tolerability of Intravenously Administered VLTS-587 in Patients with Solid Tumors and the Presence of Metastases or Primary Cancer in the Lungs.* Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 7-28-00. Not Selected for RAC Public Review: 9-7-00

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**0008-410 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous**

Vieweg, Johannes; Duke University, Durham, North Carolina; *A Safety and Feasibility Study of Active Immunotherapy in Patients with Metastatic Prostate Carcinoma using Stably Matured Dendritic Cells Transfected Amplified Autologous Tumor RNA.*

NIH/OBA Receipt Date: 8-22-00. Not Selected for RAC Public Review: 9-12-00  
Closed to enrollment: 2-13-03

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**0009-411 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Disorders/Restenosis/In Vivo/Vascular Smooth Muscle Cells/Cationic Liposome Complex/Inducible Nitric Oxide Synthase (iNOS) cDNA/Barath® Intramural Local Drug Delivery Device (Infiltrator®)**

Kuntz, Richard E.; Brigham and Women's Hospital, Boston, Massachusetts; *Restenosis Gene Therapy Trial - Phase I Study (REGENT I)*. Sponsor: Cardion AG

NIH/OBA Receipt Date: 9-20-00. Publicly Reviewed at the December 2000 RAC meeting  
Closed: 10-01 (no individuals enrolled)

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**0009-412 (Open) Gene Therapy/Phase III/Cancer/Squamous Cell Carcinoma of the Head and Neck (SCCHN)/Tumor Suppressor Gene/In Vivo/Adenovirus Serotype 5/p53 cDNA/Intratumoral Injections [INGN 201 (Ad5CMV-p53)-T302]**

Haigentz, Missak; Montefiore Medical Center, Albert Einstein College of Medicine of Yeshiva University, Bronx, New York; Nemunaitis, John J.; Mary Crowley Medical Research Center, Dallas, Texas; Hamm, John T.; Louisville Oncology, Norton Healthcare, Inc., Louisville, Kentucky; Spiro, Jeffrey; University of Connecticut Health Center, Farmington, Connecticut; Van Echo, David; University of Maryland, Baltimore, Maryland; Yoo, George; Wayne State University, Detroit, Michigan; Cobb, Patrick; Billings Oncology Associates, Billings, Montana; Brockstein, Bruce; Evanston Hospital, Evanston, Illinois; Flood, William; The Milton S. Hershey Medical Center, Hershey, Pennsylvania; Kreml, Greg; University Hospital, Oklahoma City, Oklahoma; Goodwin, W. Jarrard; University of Miami Hospital and Clinics, Miami, Florida; Trask, Douglas; University of Iowa Hospitals and Clinics, Iowa City, Iowa; Zitsch, Robert; University of Missouri Health Care, Columbia, Missouri; Breaux, Randall; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Cullen, Kevin; Georgetown University Medical Center, Washington, D.C.; Hrushesky, William; J. M., Dorn Veterans Affairs Medical Center, Columbia, South Carolina; Clayman, Gary; University of Texas, M.D. Anderson Cancer Center, Houston, Texas; Nemecek, Andrew; Tulane Cancer Center, New Orleans, Louisiana; Guthrie, Troy H., Jr.; University of Florida, Jacksonville, Florida; Trask, Douglas; University of Iowa Health Care, Iowa City, Iowa; Arquette, Matthew; Washington University School of Medicine, St. Louis, Missouri; Villaret, Douglas; University of Florida, Gainesville, Florida; Levine, Marshall; Greater Baltimore Medical Center, Baltimore, Maryland; Kane, Madeleine; University of Colorado Health Sciences Center, Denver, Colorado; Arseneau, James C.; New York Oncology Hematology, P.C., Albany, New York; Berman, Barry S.; Cancer Centers of Florida, Orlando, Florida; Lydiatt, Daniel; Methodist Hospital, Omaha, Nebraska; McGuire, Elizabeth; New Mexico VA Health Care System, Albuquerque, New Mexico; Raju, Robert; Dayton Oncology & Hematology, PA, Kettering, Ohio; Richards, Donald; Tyler Cancer Center, Tyler, Texas; and Weisman, Robert; University of California, San Diego, La Jolla, California; *A Phase III, Multi-Center, Open-Label, Randomized Study to Compare the Effectiveness and Safety of Intratumoral Administration of INGN 201 in Combination with Chemotherapy Versus Chemotherapy Alone in 288 Patients with Recurrent Squamous Cell Carcinoma of the Head and Neck (SCCHN)*. Sponsor: Aventis Pharmaceuticals - Gencell Division

NIH/OBA Receipt Date: 9-22-00. Not Selected for RAC Public Review: 10-13-00

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**0009-413 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/Tyrosinase cDNA/Intra-lymphnodal Injection**

Weber, Jeffrey; Keck/USC School of Medicine, USC Norris Center and Hospital, Los Angeles, California; Smith II, John W.; Earl A. Chiles Research Institute Providence Portland Medical Center, Portland, Oregon; and Johnson, Denise; Stanford University, Stanford, California; *A Phase I Dose Ranging Safety Study Using Intra-Nodal Delivery of a Plasmid DNA (Synchrotope TA2M) in Adult Stage IV Melanoma Patients*.

NIH/OBA Receipt Date: 9-29-00. Not Selected for RAC Public Review: 11-7-00  
Study complete: 4-4-02

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**0010-414 (Open) Gene Therapy/Phase I-II/Cancer/Hepatocellular Carcinoma/In Vitro/Autologous Dendritic Cells/Adenovirus/Alpha Fetoprotein/Intravenous Infusion**

Economou, James S.; Glapsy, John A.; and McBride, William H.; UCLA Medical Center, Los Angeles, California; *A Phase I/II Trial Testing Alpha-Fetoprotein (AFP) Genetic Immunization in Hepatocellular Carcinoma*.

NIH/OBA Receipt Date: 10-2-00. Not Selected for RAC Public Review: 10-23-00

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**0010-415 (Open) Gene Therapy/Phase II/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Ch1-DOPE/E1A/Intraperitoneal Administration**

Ueno, Naoto; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase II Study of Intraperitoneal E1A-Lipid Complex for Patients with Advanced Epithelial Ovarian Cancer without HER-2/neu Overexpression*. Sponsor: Targeted Genetics Corporation

NIH/OBA Receipt Date: 10-6-00. Not Selected for RAC Public Review: 11-2-00

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**0010-416 (Open) Gene Therapy/Phase II/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chl-DOPE/E1A/Intraperitoneal Administration**

Ueno, Naoto; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase II Study of Intraperitoneal E1A-Lipid Complex for Patients with Advanced Epithelial Ovarian Cancer with HER-2/neu Overexpression*. Sponsor: Targeted Genetics Corporation

NIH/OBA Receipt Date: 10-6-00. Not Selected for RAC Public Review: 11-2-00

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**0010-417 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Colorectal/Hepatic Metastasis/Dominant Negative Mutation/In Vivo/Retrovirus/dnG1 Cyclin/Hepatic Arterial Infusion**

Lenz, Heinz-Josef; Norris Cancer Center, University of Southern California, Los Angeles, California; *Tumor Site Specific Phase I/II Evaluation of Safety and Efficacy of Hepatic Arterial Infusion of a Matrix-Targeted Retroviral Vector Bearing a Dominant Negative Cyclin G1 (dnG1) Construct as Treatment for Colorectal Carcinoma Metastatic to Liver*.

NIH/OBA Receipt Date: 10-13-00. Publicly Reviewed at the December 2000 RAC meeting

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**0010-418 (Open) Gene Therapy/Phase II/Cancer/Prostate/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Percutaneous Transperineal Intraprostatic Injection**

Pollack, Alan; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Randomized Phase II Study of Ad5CMV-p53 plus Radioactive Seed Implant vs Seed Implant Alone for PSA Relapse after External Beam Radiotherapy for Prostate Cancer*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 10-16-00. Not Selected for RAC Public Review: 11-7-00

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**0010-419 (Open; RAC Reviewed with Recommendations) Gene Therapy/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/fhVII/hFc cDNA/Intratumoral Injection**

Deisseroth, Albert; Sidney Kimmel Cancer Center, San Diego, California; *Intratumoral Injections of a Replication-Incompetent Adenoviral Vector Encoding a Factor VII Immunoconjugate to Induce a Cytolytic Immune Response against Melanoma Tumors: A Pilot Trial*.

NIH/OBA Receipt Date: 10-18-00. Publicly Reviewed at the December 2000 RAC meeting

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**0010-420 (Closed) Gene Therapy/Phase I-II/Other Disorders/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration**

Crystal, Ronald G; Cornell University Medical College, New York, New York; and Rosengart, Todd; Evanston Northwestern Healthcare, Evanston, Illinois; *Phase I/II, Prospective, Placebo Controlled, Randomized Assessment of Direct Administration of a Replication Deficient Adenovirus Vector (Ad<sub>CMV</sub>/VEGF121.1) Containing the VEGF121 cDNA to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease as an Adjunct to Coronary Bypass Surgery*.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00  
Closed, never initiated, no individuals enrolled: 5-14-03

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**0010-421 (Open) Gene Therapy/Phase I/Other Disorders/Ulcer/In Vivo/Adenovirus/Serotype 5/Platelet Derived Growth Factor (PDGF) cDNA/Intra Ulcer Administration**

Mozingo, David; University of Florida College of Medicine, Gainesville, Florida; Mulder, Gerit D.; University of California, San Diego Medical Center, San Diego, California; and Tallis, Arthur; Associated Foot and Ankle Specialists, Phoenix, Arizona; *A Dose Escalating Phase I Study of AdPDGF-B/GAM in the Treatment of Diabetic Ulcers of the Lower Extremity*. Sponsor: Selective Genetics, Inc.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

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**0010-422 (Open) Gene Therapy/Phase I/Infectious Diseases/HIV-1/Replication Inhibition/Single Chain Antibody Gene/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/sFvhtat2 ant-HIV-1 Tat Protein Antibody/Intravenous Infusion**

Marasco, Wayne; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Pilot Study to Evaluate the Safety and Effects of Autologous Lymphocytes Transduced with a Human Single-Chain Antibody Directed against the HIV-1 Tat Protein in HIV-1 Infected Human Subjects with Advanced Disease*.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

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**0010-423 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Monogenic Diseases/Junctional Epidermolysis Bullosa/In Vitro/Autologous Keratinocytes/Retrovirus/Laminin 5-beta3 cDNA/Skin Graft**

Kimball, Alexa B.; Stanford University Medical Center; *Laminin 5 Beta 3 Gene Therapy for Junctional Epidermolysis Bullosa*.

NIH/OBA Receipt Date: 10-18-00. Publicly Reviewed at the December 2000 RAC meeting

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**0010-424 (Open) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/Plasmid DNA/Poloxamer 188/Del-1 cDNA/Intramuscular Injection**

Hinohara, Tomoaki; Cardiovascular Medicine and Coronary Interventions, Redwood City, California; Litt, Marc R.; Jacksonville Heart Center, Jacksonville, Florida; Karlsberg, Ronald P.; Cardiovascular Research Institute, Beverly Hills, California; Schaer, Gary L.; Rush-Presbyterian-St. Luke's Medical Center, Chicago, Illinois; Piana, Robert; Vanderbilt University Medical Center, Nashville, Tennessee; Jaff, Michael; The Heart and Vascular Institute of New Jersey, Morristown, New Jersey; and Rajagopalan, Sanjay; University of Michigan Health System, Ann Arbor, Michigan; *Developmentally Regulated Endothelial Locus (Del-1) Gene Medicine (VLTS-589) A Phase I Multi-Center, Open-Label, Single-Dose Escalation Clinical Safety Trial of VLTS-589 for the Treatment of Patients with Peripheral Arterial Disease*. Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

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**0010-425 (Under Review) Gene Therapy/Phase I/Other Disorders/Bone Fracture/In Vivo/Plasmid DNA/Collagen Sponge/Parathyroid Hormone cDNA/Bone Administration**

Goulet, James Alan; University of Michigan, Ann Arbor, Michigan; *A Prospective, Randomized Study to Assess the Safety of MAT-100 in Open Tibia Fractures Requiring an Intramedullary Rod (Phase I)*. Sponsor: EBI, L.P./Biomed, Inc. and Selective Genetics, Inc.

NIH/OBA Receipt Date: 10-18-00. Review at a RAC meeting pending; sponsor has requested postponement of public review.

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**0010-426 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Adenovirus Serotype 5/Replication-Competent Virus/Osteocalcin Promoter/Intratumoral Injection**

Gardner, Thomas A.; Indiana School of Medicine, Indianapolis, Indiana; *A Phase I Study of Intratumoral Injections of OCaP1 for Metastatic or Locally Recurrent Prostate Cancer, Part 1: Dose Finding, Part 2: Index Lesion Escalation*. Sponsor: DirectGene, Inc.

NIH/OBA Receipt Date: 10-18-00. Publicly Reviewed at the December 2000 RAC meeting

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**0010-427 (Open) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Adenovirus Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intradermal Administration**

Crystal, Ronald G.; Cornell University Medical College, New York, New York; *Effect of Ad<sub>GV</sub>CFTR.10 on the Cystic Fibrosis Phenotype*.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

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**0010-428 (Open) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/Pro-Drug/In Vivo/Adenovirus Serotype 5/Replication-Competent Virus/Cytosine Deaminase cDNA/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection**

Kim, Jae Ho and Freytag, Svend; Henry Ford Health System, Detroit, Michigan; *Phase I Study of Intraprostatic Administration of a Replication-Competent, Oncolytic Adenovirus Using Various Vector formulations to Patients with Localized Prostate Cancer Prior to Radical Prostatectomy*.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

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**0010-429 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma (SCCHN)/Immunotherapy/In Vivo/Fowlpox Virus/B7.1 (CD 80)/ICAM-1/LFA-3/Intratumoral Injection**

Van Waes, Carter; National Institutes of Health, Bethesda, Maryland; *Phase I/Pilot Study of Intralesional Immunotherapy with a Recombinant Avipox Virus Engineered to Express a Triad of Co-stimulatory Molecules in Patients with Advanced Squamous Cell Carcinoma of the Head and Neck*.

NIH/OBA Receipt Date: 10-26-00. Not Selected for RAC Public Review: 5-10-01

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**0011-430 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Serotype-5/Interleukin-7 cDNA/Intratumoral Injection**

Dubinett, Steven M.; UCLA School of Medicine, Los Angeles, California; *A Phase I/II Trial Evaluating Intratumoral Injection of Interleukin-7 Gene Modified Autologous Dendritic Cells for the Treatment of Non-Small Cell Lung Cancer.*

NIH/OBA Receipt Date: 11-1-00. Not Selected for RAC Public Review: 11-22-00

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**0011-431 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection**

Gonzalez, Rene; University of Colorado Health Sciences Center; Whitman, Eric D.; Missouri Baptist Medical Center, The Melanoma Center of St. Louis, St. Louis, Missouri; Amatruda, Thomas; North Memorial Health Care/Hubert H. Humphrey Cancer Center, Robbinsdale, Minnesota; Morse, Michael; Duke University Medical Center, Durham, North Carolina; Atkins, Michael B.; Beth Israel Deaconess Medical Center, Boston, Massachusetts; Bedikian, Agop Y.; The University of Texas, M.D. Anderson Cancer Center, Houston, Texas; Lutzky, Jose; Mt. Sinai Comprehensive Cancer Center, Miami, Florida; Hutchins, Laura; University of Arkansas for Medical Sciences and Central Arkansas Veteran's Healthcare System, Little Rock, Arkansas; Schwarzenberger, Paul; Louisiana State University Health Sciences Center Lions Clinic, and the Medical Center of Louisiana at New Orleans, New Orleans, Louisiana; Patel, Ravi; Comprehensive Blood and Cancer Center, Bakersfield, California; Thant, Myo; Maryland Hematology/Oncology Associates, Baltimore, Maryland; Thompson John A.; University of Washington Medical Center and the Seattle Cancer Care Alliance, Seattle Washington; Galanis, Evanthia; Mayo Clinic, Rochester, Minnesota; Ernstoff, Marc; Dartmouth-Hitchcock Medical Center, Lebanon, New Hampshire; Richards, Jon M.; Oncology Specialties, S. C., Park Ridge, Illinois; Klencke, Barbara; University of California, San Francisco Comprehensive Cancer Center at Mount Zion, San Francisco, California; Hersh, Evan M.; Arizona Cancer Center, Tucson, Arizona; and Blum, Ronald; Beth Israel Medical Center, New York, New York; *A Phase II Study of High-Dose Allovectin-7 in Patients with Advanced Metastatic Melanoma.* Sponsor: Vical Inc.

NIH/OBA Receipt Date: 11-16-00. Not Selected for RAC Public Review: 12-7-00  
Closed to enrollment: 4-23-04

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**0011-432 (Closed) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE.Vical-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection**

Gleich, Lyon, University of Cincinnati Medical Center, Cincinnati, Ohio; Khan, Mumtaz, Henry Ford Health System, Detroit, Michigan; Wolf, Gregory T., University of Michigan Health System, Ann Arbor, Michigan; Stenson, Kerstin M., The University of Chicago, Chicago, Illinois; Weinstein, Gregory, The Hospital of the University of Pennsylvania, Philadelphia, Pennsylvania; Lavertu, Pierre, Case Western University, University Hospitals of Cleveland, Cleveland, Ohio; Carroll, William, University of Alabama-Birmingham, Birmingham, Alabama; Hanna, Ehab, University of Arkansas for Medical Sciences, Little Rock, Arkansas; McCaffrey, Thomas, H. Lee Moffitt Cancer Center and Research Institute, Tampa, Florida; and Friedlander, Paul, Louisiana State University Health Sciences Center, New Orleans, Louisiana; *A Phase II Study of Safety and Efficacy of Allovectin-7 Immunotherapy for the Treatment of Primary Resectable Squamous Cell Carcinoma of the Oral Cavity or Oropharynx.* Sponsor: Vical Inc.

NIH/OBA Receipt Date: 11-16-00. Not Selected for RAC Public Review: 12-7-00  
Closed to enrollment: 6-23-03

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**0011-433 (Open) Gene Marking/Cancer/Acute or Chronic Myelogenous Leukemia, Non-Hodgkin's Lymphoma, Myelodysplastic Syndrome/In Vitro/Epstein-Barr Virus Specific Allogeneic Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplantation**

Brenner, Malcolm, Baylor College of Medicine, Houston, Texas; *A Phase I Trial Evaluating the Use of RFT5-dgA to Deplete Alloreactive Cells Prior to Haploidentical Stem Cell Transplantation.*

NIH/OBA Receipt Date: 11-27-00. Not Selected for RAC Public Review: 12-15-00

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**0011-434 (Open) Gene Therapy/Phase I/Cancer/Various Types/Immunotherapy/In Vitro/Allogeneic Fibroblasts/Lethally Irradiated/Plasmid DNA/Interleukin-2 cDNA/Intratumoral Injection**

Sobol, Robert E., Sidney Kimmel Cancer Center, San Diego, California; *A Phase I Study of Intra-Tumoral Injection with Allogeneic Fibroblasts Genetically Modified to Secrete IL-2 in Patients with Cancer Who Have Failed Standard Therapy*

NIH/OBA Receipt Date: 11-27-00. Not Selected for RAC Public Review: 12-22-00

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**0011-435 (Closed) Gene Therapy/Phase I-II/Cancer/Myeloma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection**

Borrello, Ivan, Johns Hopkins University School of Medicine, Baltimore, Maryland; *Vaccination in Peripheral Stem Cell Transplant Setting for Multiple Myeloma: The Use of Autologous Tumor Cells with an Allogeneic GM-CSF Producing Bystander Cell Line.* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 11-29-00. Not Selected for RAC Public Review: 12-28-00  
Closed to further enrollment: 2-13-04

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**0012-436 (Open) Gene Therapy/Phase I/Cancer/Glioma/Immunotherapy/In Vitro/Autologous Fibroblasts/In Combination with Untransduced Autologous Tumor and Dendritic Cells/Interleukin-4 cDNA/Intradermal Injection**

Okada, Hideho, University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; *Gene Therapy of Malignant Gliomas: A Pilot Study of Vaccination with Autologous Glioma-Lysate and Dendritic Cells Admixed with IL-4 Transduced Fibroblasts to Elicit an Immune Response.*

NIH/OBA Receipt Date: 12-5-00. Not Selected for RAC Public Review: 12-27-00

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**0012-437 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Adenovirus/Serotype 5/CD40 Ligand cDNA/Intratumoral Injection**

Harvey, Ben-Gary and Crystal, Ronald G., New York Presbyterian Hospital-Weill Medical College, Cornell University, New York, New York; *In Vivo Transfer of the CD40 Ligand Gene to Primary Lung Tumors to Activate Dendritic Cells and Induce Anti-Tumor Immunity.*

NIH/OBA Receipt Date: 12-7-00. Not Selected for RAC Public Review: 12-28-00

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**0012-438 (Open) Gene Therapy/Cancer/Head and Neck Squamous Cell Carcinoma/Oncogene Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intratumoral Injection**

Arseneau, James C., Albany Regional Cancer Center, Albany, New York; Berman, Barry S., Cancer Centers of Florida, Orlando, Florida; Anthony, Stephen P., Cancer Care Northwest, Spokane, Washington; Richards, Donald A., Tyler Cancer Center, Tyler, Texas; and Nemunaitis, John and Senzer, Neil, US Oncology, Dallas, Texas; *A Multicenter, Phase II Study of Intratumoral Injections of E1A-Lipid Complex and Re-Irradiation for Treatment of Patients with Recurrent Head and Neck Squamous Cell Carcinoma.* Sponsor: Targeted Genetics

NIH/OBA Receipt Date: 12-22-00. Not Selected for RAC Public Review: 3-2-01

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**0012-439 (Closed) Gene Therapy/Phase I/Other/Peripheral Artery Disease/In Vivo/Ischemic Lower Limb/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Intramuscular Injection**

Crystal, Ronald G., Weill Medical College, Cornell University, New York, New York; *Gene Therapy in Conjunction with Operative Bypass Grafting for Severe Peripheral Vascular Ischemia in Individuals with Insulin-Dependent Diabetes.*

NIH/OBA Receipt Date: 12-26-00. Not Selected for RAC Public Review: 1-16-01

Closed, never initiated, no individuals enrolled: 11-20-03

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**0101-440 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Murine gp75 Melanoma Antigen/Intramuscular Injection**

Wolchok, Jedd D., Memorial Sloan Kettering Cancer Center, New York, New York; *Phase I Study of gp75 DNA Vaccine in Patients with AJCC Stage III and IV Melanoma.* Sponsor: ImClone Systems, Inc.

NIH/OBA Receipt Date: 1-3-01. Not Selected for RAC Public Review: 1-24-01

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**0101-441 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Vaccinia Virus/B7.1 (CD80)/ICAM-1/LFA-3/Intratumoral Injection**

Kaufman, Howard L., Columbia University, New York, New York; *A Phase I Trial of Intralesional rV-TRICOM Vaccine in the Treatment of Malignant Melanoma.*

NIH/OBA Receipt Date: 1-4-01. Not Selected for RAC Public Review: 1-26-01

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**0101-442 (Closed) Gene Therapy/Phase I-II/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration**

Crystal, Ronald G., Weill Medical College, Cornell University, New York, New York; and Rosengart, Todd K., Evanston Northwestern Healthcare, Evanston, Illinois; *Phase I/II, Prospective Placebo Controlled, Randomized Assessment of Adenoviral Mediated VEGF121 cDNA Myocardial Angiogenesis Therapy as an Adjunct to Individuals with Diffuse Coronary Artery Disease Undergoing Repeat Coronary Artery Bypass Surgery.*

NIH/OBA Receipt Date: 1-9-01. Not Selected for RAC Public Review: 1-30-01

Closed: 9-30-03, no participants enrolled.

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**0101-443 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/Antisense TAR/Antisense tat-rev/In Vitro/CD34+ Cells/Intravenous**

Laurence, Jeffrey C., Cornell University Medical College, New York, New York; *Evaluation of the Safety and Effects of ex vivo Modification and Re-Infusion of CD34+ Cells by an Antisense Construct Against HIV-1 in a Retrovirus Vector*. Sponsor: Enzo Therapeutics, Inc.

NIH/OBA Receipt Date: 1-10-01. Publicly Reviewed at the March 2001 RAC meeting

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**0101-444 (Open) Gene Therapy/Phase I/Coronary Artery Disease/In Vivo/Muscle Cells/Plasmid DNA/Poloxamer 188/Del-1 cDNA/Retrograde Intravenous (rIV) Injection into the Heart**

Dreiling, Roger J., Cardiovascular Consultants of Oregon, Corvallis, Oregon; *A Phase I Multi-Center, Open-Label, Single-Dose Escalation Clinical Trial of VLTS-589 for Treatment of Patients with Coronary Artery Disease*. Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 4-20-01

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**0101-445 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Head and Neck Squamous Cell Carcinoma/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection**

Clayman, Gary, The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Clinical Protocol for Wild Type p53 Gene Induction in Premalignancies of Squamous Epithelium of the Oral Cavity Via an Adenoviral Vector*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 1-10-01. Publicly Reviewed at the March 2001 RAC meeting

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**0101-446 (Open) Gene Therapy/Phase I/Monogenic Disease/Severe Combined Immune Deficiency Due to JAK3 Deficiency/In Vitro/Autologous CD34+ Cells from Peripheral Blood or Bone Marrow/Retrovirus/JAK3 cDNA/Intravenous Infusion**

Sorrentino, Brian P. and Cunningham, John M., St. Jude Children's Research Hospital, Memphis, Tennessee; and Buckley, Rebecca, Duke University School of Medicine, Durham, North Carolina; *Transplantation of Gene-Corrected Autologous CD34+ Hematopoietic Stem Cells in Previously Transplanted Patients with JAK3 Deficiency and Persistent Humoral Immune Defects*.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

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**0101-447 (Open) Gene Therapy/Phase I/Cancer/Prostate Cancer/In Vivo/Dendritic Cells/Adenovirus/Serotype 5/PSA cDNA/Subcutaneous Injection**

Lubaroff, David, University of Iowa, Iowa City, Iowa; *Phase I Study of Adenovirus/PSA Vaccine in Men with Metastatic Prostate Cancer*.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

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**0101-448 (Open) Gene Therapy/Phase II-III/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection**

Swisher, Stephen, University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase II/III, Multi-Center, Open-Label, Randomized Study to Compare the Effectiveness and Safety of Intralesional Administration of RPR/INGN 201 in Combination with Taxotere<sup>®</sup> and Carboplatin and Radiotherapy Versus Taxotere<sup>®</sup> and Carboplatin and Radiotherapy Alone in Patients with Locally Advanced Unresectable Non-Small Cell Lung Cancer (NSCLC)*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

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**0101-449 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection**

Miles, Brian J., Baylor College of Medicine, Houston, Texas; *Phase I Study of Adenoviral Vector Delivery of the IL-12 Gene in Men with Local Recurrence of Prostate Cancer After Irradiation Therapy*.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

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**0101-450 (Closed) Gene Therapy/Phase II/Cancer/Prostate Cancer/Vector Directed Cell Lysis/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Replication Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intratumoral Injection**

DeWeese, Theodore, Johns Hopkins Oncology Center, Baltimore, Maryland; Roach III, Mack, University of California, San Francisco, San Francisco, California; and Michalski, Jeff, Washington University Medical School, Saint Louis, Missouri; *A Phase II Randomized Comparison Study of an Intraprostatic Injection of CV7606 Followed by External Beam Radiotherapy (EBRT) Versus EBRT Alone in Patients with Intermediate Risk, Clinically Localized Prostate Cancer.* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01  
Study was never initiated

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**0101-451 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Vector Directed Cell Lysis/In Vivo/Adenovirus/Serotype 5/Replication Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intravenous Injection**

Small, Eric, University of California, San Francisco, San Francisco, California; *A Randomized, Placebo Controlled Phase II Study of an Intravenous Injection of CV787, a Prostate-Specific Antigen Oncolytic Adenovirus, Plus Weekly Docetaxel in Patients with Metastatic Hormone Refractory Prostate Cancer.* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01  
Study was never initiated

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**0101-452 (Closed) Gene Therapy/Phase IIb-III/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration**

Grines, Cindy L., William Beaumont Hospital, Royal Oak, Michigan; Bethala, Vasanth, Medical Research Institute, Slidell, LA; Erenrich, Norman, Florida Cardiovascular Research, Atlantis, FL; Gammon, Roger, Austin Heart, Austin, TX; Henry, Timothy, Abbott Northwestern Hospital, Minneapolis, MN; Licandro, Rudolph, Louisville Cardiology Medical Group, Louisville, KY; Saucedo, Jorge, University of Arkansas for Medical Sciences, Little Rock, AR; Tonkon, Melvin, Anaheim Heart and Research Institute, Santa Ana, CA; Watkins, Matthew, The University of Vermont, Burlington, VT; Grossman, P. Michael, The University of Michigan Health System, Ann Arbor, Michigan; Butman, Samuel, University Medical Center, University of Arizona, Tucson, Arizona; Churchill, David A., Washington Regional Medical Center, Fayetteville, Arkansas; Conn, Eric H., The Chattanooga Heart Institute, Chattanooga, Tennessee; Coppola, John T., Saint Vincent Catholic Medical Centers of New York, New York, New York; Fuchs, Shmuel, Washington Hospital Center, Washington, D.C.; Ghali, Jalal K., Cardiac Centers of Louisiana, Shreveport, Louisiana; Hodes, Zachary I., The Care Group, LLC, Indianapolis, Indiana; Nadar, Venkatesh K., Heritage Cardiology Associates, Camp Hill, Pennsylvania; Rowe, Steven K., Heartland Regional Medical Center, St. Joseph, Missouri; Brennan, Theresa, University of Iowa Healthcare, Iowa City, Iowa; Browne, Jr., Kevin F., Watson Clinic LLP, Lakeland, Florida; Dib, Nabil, Arizona Heart Institute, Phoenix, Arizona; Ellis, Stephen, Cleveland Clinic Foundation, Cleveland, Ohio; Hart, Kevin, Stucky Research Center, Fort Wayne, Indiana; Iskandrian, Ami E., University of Alabama at Birmingham, Birmingham, Alabama; Kleiman, Neal S., Baylor College of Medicine, Houston, Texas; Marmur, Jonathan, Mount Sinai Hospital, New York, New York; Marshall, J. Jeffrey, Crawford Long Hospital, Atlanta, Georgia; Penny, William F., San Diego VA Medical Center, San Diego, California; Pepine, Carl J., University of Florida, Gainesville, Florida; Saenz, Carlos and Taussig, Andrew, Florida Hospital, Orlando, Florida; Schaer, Gary L., Rush-Presbyterian St. Luke's Medical Center, Chicago, Illinois; Sequeira, Rafael F., University of Miami-Jackson Memorial Hospital, Miami, Florida; Baran, Kenneth W., United's John Nasseff Heart Hospital, Saint Paul, Minnesota; Helmer, Gregory A., Minnesota Heart Clinic, P.A., Edina, Minnesota; Mendelsohn, Farrell O., Cardiology, P.C., Birmingham, Alabama; Moran, John F., Loyola University Medical Center, Maywood, Illinois; Sanborn, Timothy, Evanston Northwestern Healthcare, Evanston, Illinois; Sharaf, Barry L., Rhode Island Hospital, Providence, Rhode Island; Moreyra, Abel E., Robert Wood Johnson Medical School, New Brunswick, New Jersey; Cohen, Eric, Cardiovascular Associates, P.C., Birmingham, Alabama; Lee, Joon, University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; Levine, Glenn, Houston VA Medical Center, Houston, Texas; Lopez, John, University of Chicago Medical Center, Chicago, Illinois; McGrew, Frank, III, The Stern Cardiovascular Center, Memphis, Tennessee; Thai, Hoang, Southern Arizona Veterans Affairs Health Care System, Tucson, Arizona; Zabalgotia, Miguel, The University of Texas Health Science Center at San Antonio, San Antonio, Texas; Laham, Roger, Beth Israel Deaconess Medical Center, Boston, Massachusetts; Murphy, Patrick, L., The Heart Group, PC, Mobile, Alabama; Rade, Jeffrey J., Johns Hopkins University School of Medicine, Baltimore, Maryland; Simari, Robert D., Mayo Clinic, Rochester, Minnesota; Savage, Michael P., Jefferson Heart Institute, Philadelphia, Pennsylvania; Zoble, Robert G., James A. Haley Veterans Hospital, Tampa, Florida; Kellett, Mirlle, Maine Medical Center, Portland, Maine; Moreyra, Abel, Robert Wood Johnson University Hospital, New Brunswick, New Jersey; Niles, Nathaniel, Dartmouth-Hitchcock Medical Center, Lebanon, New Hampshire; Ohman, Erik, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina; Murray, Conrad, Global Cardiovascular Associates, Inc., Las Vegas, Nevada; Furman, Mark I., University of Massachusetts Medical Center, Worcester, Massachusetts; Grossman, P. Michael, University of Michigan Health Systems, Ann Arbor, Michigan; Chronos, Nicholas, Atlanta Cardiology Research Institute, Atlanta, Georgia; Mahmud, Ehtisham, University of California, San Diego, Medical Center, San Diego, California; Molk, Barry, Aurora Denver Cardiology Associates, P.C., Denver, Colorado; Bhoopalani, Vishwajeth, Nebraska Heart Institute, Lincoln, Nebraska; West, Andrew J., Heart & Vascular Institute of Texas, PA, San Antonio, Texas; Corbelli, John C., Buffalo Cardiology & Pulmonary Associates PC, Williamsville, New York; Lieberman, Scott, East Texas Medical Center, Tyler, Texas; Goldberg, Steven, University of Washington Medical Center, Seattle, Washington; and Durkin, Raymond A., Lehigh Valley Hospital, Allentown, Pennsylvania; *A Multicenter, Randomized, Double-Blind, Placebo Controlled, Dose-Response Study to Evaluate the Efficacy and Safety of Ad5.1FGF-4 in Patients with Stable Angina.* Sponsor: Berlex Laboratories.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01  
Closed to enrollment: 05-14-04

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**0101-453 (Closed) Gene Therapy/Phase I/Cancer/Glioblastoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Interferon-beta cDNA/Stereotactic Injection**

Eck, Stephen L., University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; Rosenfeld, Steven S., University of Alabama at Birmingham, Birmingham, Alabama; Chiocca, E. Antonio, Massachusetts General Hospital, Boston, Massachusetts; Hamilton, Allan, University of Arizona, Tucson, Arizona; and Lillehei, Kevin, University of Colorado Health Sciences Center, Denver, Colorado; *A Multi-Center, Open Label, Two Part, Dose Escalation Study to Determine the Tolerability of Interferon-beta Gene Transfer in the Treatment of Recurrent or Progressive Glioblastoma Multiforme*. Sponsor: Biogen Idec, Inc..

NIH/OBA Receipt Date: 1-12-01. Not Selected for RAC Public Review: 1-31-01  
Closed: 7-28-04

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**0101-454 (Open) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection**

Yoo, George H., Wayne State University, Detroit, Michigan ; Taylor, Sarah A., The University of Kansas Medical Center, Kansas City, Kansas; and Valentino, Joseph; University of Kentucky, Lexington, Kentucky; *Phase II Trial of Surgery with Perioperative RPR/INGN 201 (Ad5CMV-p53) Gene Therapy Followed by Chemoradiotherapy for Advanced Resectable Squamous Cell Carcinoma of the Oral Cavity and Oropharynx*. Sponsor: Southwest Oncology Group.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

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**0101-455 (Open) Gene Therapy/Phase II/Cancer/Breast/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection**

Cristofanilli, Massimo, The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Phase II, Single Arm, Single Institution Clinical Trial of Docetaxel and Doxorubicin in Combination with Local Administration of Ad5CMV-p53 (RPR/INGN-201) in Locally Advanced Breast Cancer (LABC)*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 1-16-01. Not Selected for RAC Public Review: 1-31-01

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**0101-456 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD 80)/ICAM-1/LFA-3/GM-CSF/Intramuscular or Intradermal Injection**

von Mehren, Margaret, Fox Chase Cancer Center, Philadelphia, Pennsylvania; *Phase I Study of a Recombinant Fowlpox Vaccine rF-CEA(6D)/TRICOM alone or with GM-CSF in Patients with Advanced CEA Expressing Adenocarcinoma*.

NIH/OBA Receipt Date: 1-25-01. Not Selected for RAC Public Review: 2-27-01

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**0101-457 (Closed) Gene Therapy/Phase I/Cancer/Soft Tissue Sarcoma/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection**

Hanna, Nader, University of Kentucky Chandler Medical Center, Lexington, Kentucky; Nemunaitis, John, US Oncology, Dallas, Texas; Sandler, Alan B., Vanderbilt University, Nashville, Tennessee; Vijayakumar, Srinivasan and Warso, Michael, University of Illinois at Chicago, Chicago, Illinois; Mundt, Arno, University of Chicago, Chicago, Illinois; and Richards, Donald A., Tyler Cancer Center, Tyler, Texas; *An Open-Label, Phase I, Dose-Escalation Study of TNFerade™ Biologic with Radiation Therapy as an Adjunct to Surgery or for Palliation of Soft Tissue Sarcoma of the Extremities*. Sponsor: GenVec.

NIH/OBA Receipt Date: 1-29-01. Not Selected for RAC Public Review: 2-26-01  
Enrollment completed, long-term follow-up continues: 1-15-04

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**0102-458 (Open) Gene Therapy/Phase II/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Canarypox Virus/Carcinoembryonic Antigen/B 7.1 (CD80)/Intramuscular and Intradermal Injections**

Kaufman, Howard L., Columbia University, New York, New York; von Mehren, Margaret, Fox Chase Cancer Center, Philadelphia, Pennsylvania; Conry, Robert M., The University of Alabama at Birmingham, Birmingham, Alabama; Marshall, John, Georgetown University Medical Center, Washington D.C.; Heim, William J., Hematology & Oncology Associates of Northeastern PA, Dunmore, Pennsylvania; Lenz, Heinz-Josef, University of Southern California/Norris Comprehensive Cancer Center, Los Angeles, California; Kindler, Hedy, The University of Chicago, Chicago, Illinois; Garrett, Christopher, H. Lee Moffitt Cancer Center, Tampa, Florida; Urba, Walter, Providence Portland Medical Center, Portland, Oregon; and Benson, Al B., III; Northwestern University, Chicago, Illinois; *Pilot Phase II Study of Safety and Immunogenicity of a ALVAC-CEA/B7.1 Vaccine Administered with Chemotherapy, Alone or in Combination with Tetanus Toxoid, as Compared to Chemotherapy Alone, in Patients with Metastatic Colorectal Adenocarcinoma*. Sponsor: Aventis Pasteur Limited.

NIH/OBA Receipt Date: 2-15-01. Not Selected for RAC Public Review: 4-12-01

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**0103-459 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection**

Corman, John M., Virginia Mason Medical Center, Seattle, Washington; Dula, Eugene, West Coast Clinical Research, Van Nuys, California; and Young, Jay M., South Orange County Medical Research Center, Laguna Woods, California; *A Phase I Dose Escalation Study of Human GM-CSF Gene Transduced Irradiated Allogeneic Prostate Cancer Cell Vaccine (GVAX® Prostate Cancer Vaccine (PC-3)) in Patients with Hormone-Refractory Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 3-6-01. Not Selected for RAC Public Review: 7-27-01  
Closed: 7-07-03

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**0103-460 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/Autologous Lymphoma Cells/Adenovirus/Serotype 5/Interleukin-2 cDNA/CD40 Ligand cDNA/Subcutaneous Injection**

Takahashi, Satoshi and Brenner, Malcolm, Baylor College of Medicine, Houston, Texas; *Treatment of Chronic Lymphocytic Leukemia (CLL) with IL-2 Gene Modified and Human CD40 Ligand-Expressing Autologous Tumor Cells*.

NIH/OBA Receipt Date: 3-19-01. Not Selected for RAC Public Review: 4-6-01

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**0104-461 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Interferon-alpha/Interleukin-12 cDNA/Intratumoral Injection**

Posner, Marshall R., Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I/II Multi-Center, Open-Label, Multiple Administration Trial of the Safety, Tolerability, and Efficacy of an IFN-alpha/IL-12 Plasmid-Based Therapeutic*. Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 4-9-01. Not Selected for RAC Public Review: 4-27-01

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**0104-462 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Pro-Drug/In Vivo/Tumor Cells/Salmonella typhimurium/E. coli Cytosine Deaminase cDNA/Intratumoral Injection/Combined with 5fluorocytosine**

Nemunaitis, John J. and Cunningham, Charles, Mary Crowley Medical Research Center (US Oncology), Dallas, Texas; *A Phase I Trial of Genetically Modified Salmonella typhimurium Expressing Cytosine Deaminase (TAPET-CD, VNP20029) Administered by Intra-Tumoral Injection in Combination with 5-fluorocytosine for Patients with Advanced or Metastatic Cancer*. Sponsor: Vion Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 4-16-01. Publicly Reviewed at the June 2001 RAC meeting.

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**0104-463 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Fibroblast Growth Factor 1 cDNA/Intramuscular Injection**

Comerota, Anthony J., Toledo Hospital, Toledo, Ohio; Henry, Tim, Minneapolis Heart Institute, Minneapolis, Minnesota; and Chronos, Nicolas, Atlanta Cardiovascular Research Institute, Atlanta, Georgia; *Phase I Double Blind, Parallel-Group, Multi-Center, Gene Expression (Synthesis of FGF-1 mRNA), Safety and Tolerability Study of Increasing Single Doses of NV1FGF Administered by Intra-Muscular Injection in Patients with Severe Peripheral Artery Occlusive Disease (PAOD) Planned to Undergo Amputation Above the Ankle*. Sponsor: Aventis Pharma Recherche-Développement.

NIH/OBA Receipt Date: 4-16-01. Not Selected for RAC Public Review: 5-4-01  
Study terminated: 10-03

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**0104-464 (Open) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/E. coli Cytosine Deaminase cDNA/5-Fluorocytosine/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection**

Kim, Jae Ho and Freytag, Svend O., Henry Ford Health System, Detroit, Michigan; *Phase I Study of Combined Suicide Gene Therapy and Radiation Therapy for Locally Advanced Carcinoma of the Prostate*.

NIH/OBA Receipt Date: 4-17-01. Not Selected for RAC Public Review: 5-7-01

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**0104-465 (Open) Gene Therapy/Phase I/Monogenic Disease/Alpha-1 Antitrypsin Deficiency/In Vivo/Adeno-Associated Virus/Alpha-1 Antitrypsin cDNA/Intramuscular Injection**

Flotte, Terence R., University of Florida, Gainesville, Florida; *A Phase I Trial of Intramuscular Injection of a Recombinant Adeno-Associated Virus Alpha-1-Antitrypsin (rAAV-AT) Gene Vector to AAT-Deficient Adults*.

NIH/OBA Receipt Date: 4-18-01. Not Selected for RAC Public Review: 5-8-01

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**0104-466 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Chemoprotection/In Vivo/Cationic Liposome Complex/Cholesterol/DOTIM/Manganese Super Oxide Dismutase (MnSOD)/Intraesophageal Administration**

Belani, Chandra P., University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *Concurrent Chemotherapy (Paclitaxel and Carboplatin) and Thoracic Radiotherapy with Swallowed Manganese Superoxide Dismutase (MnSOD) Plasmid Liposome (PL) Protection in Patients with Locally Advanced Stage III Non-Small Cell Lung Cancer. A Phase I-II Study.*

NIH/OBA Receipt Date: 4-18-01. Not Selected for RAC Public Review: 5-8-01

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**0104-467 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Other/Peripheral Neuropathy/In Vivo/Endothelial Cells/Plasmid DNA/VEGF<sub>165</sub> cDNA/Intramuscular Injection**

Ropper, Allan, St. Elizabeth's Medical Center, Boston, Massachusetts; *VEGF Gene Transfer for Diabetic Neuropathy.*

NIH/OBA Receipt Date: 4-18-01. Publicly Reviewed at the June 2001 RAC meeting.

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**0104-468 (Open) Gene Therapy/Phase I-II/Coronary Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/VEGF<sub>165</sub> cDNA/Intramyocardial Injection**

Smedira, Nicholas; The Cleveland Clinic Foundation, Cleveland, Ohio; *VEGF Gene Transfer to Promote Angiogenesis in Patients with Advanced Heart Failure.*

NIH/OBA Receipt Date: 4-18-01. Not Selected for RAC Public Review: 5-8-01

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**0104-469 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other/Parkinson's Disease/In Vivo/Adeno-Associated Virus/Glutamic Acid Decarboxylase 65-67 cDNA/Intracerebral Administration**

During, Matthew J., Jefferson Medical College, Philadelphia, Pennsylvania and Kaplitt, Michael, New York Hospital-Weill Medical College of Cornell University, New York, New York; *Subthalamic GAD Gene Transfer in Parkinson Disease Patients Who Are Candidates for Deep Brain Stimulation.*

NIH/OBA Receipt Date: 4-18-01. Publicly Reviewed at the June 2001 RAC meeting.

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**0104-470 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Osteosarcoma Metastasis to Lung/Vector-Directed Cell Lysis/In Vivo/Adenovirus Serotype 5/Replication-Competent Virus/Promoter of Osteocalcin/Intravenous Injection**

Meyers, Paul A., Memorial Sloan-Kettering Cancer Center, New York, New York and Reaman, Gregory H., George Washington University School of Medicine and Children's National Medical Center, Washington, D.C.; *A Phase I/II Dose Escalation and Activity Study of Intravenous Injections of OCaP1 in Subjects with Refractory Osteosarcoma Metastatic to Lung.* Sponsor: DirectGene, Inc.

NIH/OBA Receipt Date: 4-18-01. Publicly Reviewed at the June 2001 RAC meeting.

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**0104-471 (Open) Gene Therapy/Cancer/Breast Cancer/In Vivo/Tumor Suppressor/Adenovirus/Melanoma Differentiation Associated Protein-7 cDNA/Intratutormal Injection**

Buchholz, Thomas A., M.D. Anderson Cancer Center, Houston, Texas; *A Phase I/II Dose-Escalation Trial of Intratumoral Injection with a Replication-Deficient Adenovirus Vector, Ad-mda7 (INGN 241), in Combination with Radiation Therapy in Patients with Locally Recurrent Breast Cancer.* Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 4-18-01. Not Selected for RAC Public Review: 5-8-01

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**0105-472 (Closed) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection**

Smith II, John W., Providence Portland Medical Center, Portland, Oregon; Aboulafia, David, Virginia Mason Medical Center, Seattle, Washington; Sternman, Daniel H., University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; and Jablons, David M., University of California, San Francisco, San Francisco, California; *Phase I/II Study of Vaccination with Irradiated Autologous Lung Tumor Cells Mixed with a GM-CSF Secreting Bystander Cell Line (Lung Bystander GVAX®) in Advanced Non-Small Cell Lung Cancer.* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 5-14-01. Not Selected for RAC Public Review: 6-4-01

Dosing portion of study completed, follow-up continues: 12-10-02

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**0105-473 (Open) Gene Marking/Cancer/EBV-Positive Hodgkin Disease/In Vitro/LMP2A-Specific Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Adenovirus/LMP2A cDNA/Intravenous Administration**

Gahn, Benedikt, Heslop, Helen, and Rooney, Cliona, Baylor College of Medicine, Houston, Texas; *Administration of Neomycin Resistance Gene Marked LMP2A-Specific Cytotoxic T Lymphocytes to Patients with Relapsed EBV-Positive Hodgkin's Lymphoma.*

NIH/OBA Receipt Date: 5-14-01. Not Selected for RAC Public Review: 6-4-01

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**0105-474 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid DNA/Human and Mouse Prostate Specific Membrane Antigen cDNAs/Intramuscular Injection**

Scher, Howard I., and Wolchok, Jedd, D., Memorial Sloan-Kettering Cancer Center, New York, New York; *Vaccination of Prostate Cancer Patients with Human and Mouse Specific Membrane Antigen (PSMA) DNA Vaccine: A Pilot Trial to Assess Safety and the Immune Response.*

NIH/OBA Receipt Date: 5-24-01. Not Selected for RAC Public Review: 6-14-01

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**0106-475 (Open) Gene Therapy/Phase II/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection**

Jaffee, Elizabeth M., Johns Hopkins University School of Medicine, Baltimore, Maryland; *A Safety and Efficacy Trial of Lethally Irradiated Allogeneic Pancreatic Tumor Cells Transfected with the GM-CSF Gene in Combination with Adjuvant Chemotherapy for the Treatment of Adenocarcinoma of the Pancreas.*

NIH/OBA Receipt Date: 6-11-01. Not Selected for RAC Public Review: 6-29-01

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**0106-476 (Open) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) cDNA/Aerosol Administration**

Virella-Lowell, Isabel, University of Florida, College of Medicine, Gainesville, Florida; *Evaluation of Anti-Inflammatory and Anti-Protease Pretreatment on the Delivery of Aerosolized tgAAVCF to Cystic Fibrosis Patients with Mild Lung Disease.* Sponsor: Targeted Genetics.

NIH/OBA Receipt Date: 6-11-01. Not Selected for RAC Public Review: 7-12-01

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**0106-477 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Immunotherapy/In Vivo/Fowlpox Virus/B7.1 (CD80)/ICAM-1/LFA-3/Intratumoral Injection**

Kaufman, Howard L., Columbia University, New York, New York; *Intra-Lesional rF-B7.1 Versus rF-TRICOM Vaccine in the Treatment of Metastatic Cancer.*

NIH/OBA Receipt Date: 6-12-01. Not Selected for RAC Public Review: 7-2-01

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**0106-478 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vitro/Autologous Dendritic Cells/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/GM-CSF/Intravenous**

Lyerly, H. Kim, Duke University Medical Center, Durham, North Carolina; *A Phase I Study of Active Immunotherapy with Autologous Dendritic Cells Infected with CEA-6D Expressing Fowlpox-TRICOM in Patients with Advanced or Metastatic Malignancies Expressing CEA.*

NIH/OBA Receipt Date: 6-28-01. Not Selected for RAC Public Review: 7-19-01

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**0106-479 (Open) Gene Therapy/Phase I-II/Cancer/Acute Myelogenous Leukemia/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection**

Borrello, Ivan, Johns Hopkins University School of Medicine, Baltimore, Maryland; Stock, Wendy, University of Chicago, Chicago, Illinois; Damon, Lloyd, University of California, San Francisco, San Francisco, California; and DeAngelo, Daniel, Dana Farber Cancer Institute, Boston, Massachusetts; *Vaccination in Peripheral Stem Cell Transplant Setting for Acute Myelogenous Leukemia: The Use of Autologous Tumor Cells with an Allogeneic GM-CSF Producing Bystander Cell Line.* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 6-29-01. Not Selected for RAC Public Review: 7-20-01

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**0107-480 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase IIb/Other/Peripheral Artery Disease/End Stage Renal Disease/Stenosis Prevention/In Vivo/Adenovirus/Vascular Endothelial Growth Factor D/Perivascular Collagen Collar Device**

Lawson, Jeffrey H., Duke University, Durham, North Carolina; *A Phase II, Open-Label, Ascending-Dose Study of the Safety and Efficacy of Trinam™ (EG004) in Stenosis Prevention at the Graft-Vein Anastomosis Site in Dialysis Patients.* Sponsor: Ark Therapeutics, Ltd.

NIH/OBA Receipt Date: 7-6-01. Publicly Reviewed at the September 2001 RAC meeting.

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**0107-481 (Open) Gene Therapy/Phase Ib-II/Cancer/Brain Tumors/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection**

Markert, James M., University of Alabama at Birmingham, Birmingham, Alabama; *An Open-Label, Phase Ib/II Study of the Safety, Tolerability and Efficacy of G207, a Genetically Engineered Herpes Simplex Type-1 Virus, Administered Intracerebrally to Subjects with Recurrent Malignant Glioma.* Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 7-9-01. Not Selected for RAC Public Review: 7-31-01

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**0107-482 (Open) Gene Therapy/Phase Ib-II/Cancer/Brain Tumors/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection**

Markert, James M., University of Alabama at Birmingham, Birmingham, Alabama; *Long-Term Follow-Up of the Safety and Survival of Subjects with Recurrent Malignant Glioma Who Enrolled in a Phase Ib/II Study (protocol 0107-481) of the Safety, Tolerability and Efficacy of G207, a Genetically Engineered Herpes Simplex Type-1 Virus, Administered Intracerebrally.* Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 7-9-01. Not Selected for RAC Public Review: 7-31-01

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**0107-483 (Open) Gene Therapy/Phase Ib-II/Cancer/Brain Tumors/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection**

Markert, James M., University of Alabama at Birmingham, Birmingham, Alabama; *Long-Term Follow-Up of the Safety and Survival of Subjects with Recurrent Malignant Glioma Who Enrolled in a Phase Ib/II Study (protocol 0107-481) of the Safety, Tolerability and Efficacy of G207, a Genetically Engineered Herpes Simplex Type-1 Virus, Administered Intracerebrally.* Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 7-9-01. Not Selected for RAC Public Review: 7-31-01

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**0107-484 (Closed) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vivo/Plasmid in Poly (DL-lactide-coglycolide) (PLG) Microparticles/Cytochrome P450 isoenzyme 1B1 (CYP1B1) Gene/Intramuscular Injection**

Gribben, John G., Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Open-Label Study of the Safety and Feasibility of Vaccinating Cancer Patients with Repeated Doses of ZYC300.* Sponsor: ZYCOS, Inc.

NIH/OBA Receipt Date: 7-11-01. Not Selected for RAC Public Review: 7-31-01  
Closed to further enrollment: 8-14-03

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**0107-485 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Breast/In Vitro/Autologous Bone Marrow Cells/Adenovirus/bcl-2 Dominant Negative Mutant/Bone Marrow Transplant**

Clarke, Michael F., University of Michigan, Ann Arbor, Michigan; *Purging of Autologous Stem Cell Sources with bcl-x<sub>s</sub> Adenovirus for Women Undergoing High-Dose Chemotherapy for Stage IV Breast Carcinoma.*

NIH/OBA Receipt Date: 7-11-01. Publicly Reviewed at the September 2001 RAC meeting.

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**0107-486 (Open) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus-1/Replication Inhibition/In Vitro/Autologous CD34+ Cells/Retrovirus/Hammerhead Ribozyme/Intravenous**

Mitsuyasu, Ronald, UCLA Medical Center, Los Angeles, California; Merigan, Thomas C., Jr., Stanford Medical Center, Stanford, California; Cooper, David, University of New South Wales/St. Vincent's Hospital, Sydney, Australia; Workman, Cassy, AIDS Research Initiative, Sydney, Australia; Bloch, Mark, Holdsworth House General Practice, Sydney, Australia; McFarlane, Robert, 407 Doctors, Sydney, Australia; Finlayson, Robert, Taylor Square Private Clinic, Sydney, Australia; Kelly, Mark, Albion Street Centre, Sydney, Australia; and Hoy, Jennifer, The Alfred Hospital, Melbourne, Australia; *A Randomized Phase II, Double-Blind, Controlled Trial to Evaluate the Safety and Efficacy of Autologous CD34+ Hematopoietic Progenitor Cells Transduced with Either a Delivery Gene Construct (LNL6) or LNL6 that Contains an Anti-HIV-1 Ribozyme in Patients with HIV-1 Infection.* Sponsor: Johnson & Johnson Research Pty Limited.

NIH/OBA Receipt Date: 7-11-01. Not Selected for RAC Public Review: 7-31-01

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**0107-487 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other/Age-related Macular Degeneration (AMD)/Adenovirus/Pigment-Epithelium Derived Factor (PEDF) cDNA/Intravitreal Administration**

Campochiaro, Peter A., Johns Hopkins University School of Medicine, Baltimore, Maryland; Klein, Michael, Oregon Health & Science University, Portland, Oregon; Holz, Eric R., Baylor College of Medicine, Houston, Texas; Gupta, Anurag; University of California, Los Angeles, Los Angeles, California; Saperstein, David, University of Washington School of Medicine, Seattle, Washington; Azrin, Michael, University of Connecticut Health Center, Farmington, Connecticut; and Frank, Robert; Kresge Eye Institute, Detroit, Michigan; *An Open-Label, Phase I, Single Administration, Dose Escalation Study of AD<sub>CV</sub>PEDF.11D (ADPEDF) in Neovascular Age-Related Macular Degeneration (AMD)*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 7-11-01. Publicly Reviewed at the September 2001 RAC meeting.

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**0107-488 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Infectious Diseases/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Lentivirus/HIV-1/Antisense env/Intravenous**

MacGregor, Rob Roy, University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *A Phase I Open-Label Clinical Trial of the Safety and Tolerability of a Single Dose of Autologous T Cells Transduced with VRX496 in HIV Positive Patient-Subjects*. Sponsor: VIRxSYS Corporation.

NIH/OBA Receipt Date: 7-12-01. Publicly Reviewed at the September 2001 RAC meeting.

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**0107-489 (Open) Gene Therapy/Phase I/Other/Restenosis In Vivo/Plasmid DNA/Vascular Endothelial Growth Factor cDNA/Intraarterial/Angioplasty Catheter**

Losordo, Douglas W., St. Elizabeth's Medical Center, Boston, Massachusetts; *VEGF Gene Transfer to Prevent Coronary Artery Restenosis*.

NIH/OBA Receipt Date: 7-12-01. Not Selected for RAC Public Review: 7-31-01

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**0107-490 (Closed) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/Melan-A/MART-1/Intralymphnodal Injection**

Weber, Jeffrey S., University of Southern California/Norris Comprehensive Cancer Center, Los Angeles, California; Hersh, Evan M., Arizona Cancer Center, Tucson, Arizona; Smith II, John W., Providence Portland Medical Center, Portland, Oregon; and Lerner, Adam, Boston University School of Medicine, Boston, Massachusetts; *A Pilot Phase I/II Study of Intranodal Delivery of a Plasmid DNA (Synchrovax SEM Vaccine) in Stage IV Melanoma Patients*. Sponsor: CTL ImmunoTherapies Corp.

NIH/OBA Receipt Date: 7-19-01. Not Selected for RAC Public Review: 8-8-01.  
Completed: 10-02-03

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**0107-491 (Open) Gene Therapy/Phase I/Cancer/Follicular Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/ Autologous T Lymphocytes/Plasmid DNA/Electroporation/CE7R-Specific scFvFc-Zeta T Cell Receptor/Intravenous Infusion**

Press, Oliver W., University of Washington Medical Center, Seattle Washington; *A Phase I Study to Evaluate the Safety of Cellular Immunotherapy Using Genetically-Modified Autologous CD20-Specific CD8+ T Cell Clones for Patients with Relapsed CD20+ Indolent Lymphomas*.

NIH/OBA Receipt Date: 7-26-01. Not Selected for RAC Public Review: 8-15-01

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**0107-492 (Withdrawn-replaced by protocol # 0110-499) Gene Therapy/Phase I/Cancer/Liver (Hepatic) Metastases/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection**

Sung, Max W., Mount Sinai Medical Center, New York, New York; *Clinical Trial of Adenoviral Vector Delivery of the Herpes Thymidine Kinase (HSV-TK) Gene by Intratumoral Injection Followed by Intravenous Ganciclovir with Imaging of HSV1-tk Gene Expression in Patients with Hepatic Metastases from Colorectal Cancer*.

NIH/OBA Receipt Date: 7-27-01.

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**0107-493 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection**

Corman, John M., Virginia Mason Medical Center, Seattle, Washington; Simons, Jonathan, Emory University, Atlanta, Georgia; Small, Eric, University of California, San Francisco, San Francisco, California; Higano, Celestia, University of Washington, Seattle, Washington; Smith, David, University of Michigan Medical Center, Ann Arbor, Michigan; Hudes, Gary R., Fox Chase Cancer Center, Philadelphia, Pennsylvania; Centeno, Arthur, Urology San Antonio Research, San Antonio, Texas; Gittelman, Marc, South Florida Medical Research, Aventura, Florida; Steidle, Christopher, Northeast Indiana Research, Fort Wayne, Indiana; Dula, Eugene, West Coast Clinical Research, Van Nuys, California; Young, Jay M., South Orange County Medical Research Center, Laguna Woods, California; and Rowland, Kendrith M., Jr., Carle Cancer Center, Urbana, Illinois; *A Phase I/II Dose Escalation and Efficacy Trial of GVAX<sup>®</sup> Prostate Cancer Vaccine in Patients with Metastatic Hormone-Refractory Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 7-18-01. Not Selected for RAC Public Review: 8-31-01

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**0108-494 (Open) Gene Therapy/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/In Vitro/Autologous CD34+ Cells from Cord Blood or Bone Marrow/Retroviral Vector/ $\gamma$ cDNA/Intravenous Infusion**

Weinberg, Kenneth I., Children's Hospital of Los Angeles, University of Southern California School of Medicine, Los Angeles, California; *Gene Transfer of the  $\gamma$  cDNA into CD34+ Hematopoietic Cells of Infants or Children with X-Linked Severe Combined Immune Deficiency (X-SCID)*.

NIH/OBA Receipt Date: 8-27-01. Not Selected for RAC Public Review: 9-17-01

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**0108-495 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/DF3/MUC1/B7.1 (CD 80)/ICAM-1/LFA-3/Intradermal Injection**

Eder, Joseph Paul, Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Trial of Recombinant Vaccinia Viruses that Express DF3/MUC1 and TRICOM (B7.1, ICAM-1, and LFA-3) in Patients with Metastatic Adenocarcinoma of the Breast*.

NIH/OBA Receipt Date: 8-27-01. Not Selected for RAC Public Review: 3-6-02

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**0108-496 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Plasmid DNA/Electroporation/IL13R  $\alpha$  2-Specific scFvFc-Zeta T Cell Receptor/Intracavity Administration**

Jensen, Michael, City of Hope National Medical Center, Duarte, California; *Pilot Feasibility and Safety Study of Cellular Immunotherapy for Recurrent/Refractory Malignant Glioma using Genetically Modified Autologous CD8+ T Cell Clones*.

NIH/OBA Receipt Date: 8-30-01. Not Selected for RAC Public Review: 9-21-01

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**0109-497 (Open) Gene Therapy/Phase II/Cancer/MUC-1 Expressing Prostate Cancer/Immunotherapy/In Vivo/Vaccinia Virus/MUC-1/Interleukin-2/Subcutaneous Injection**

Pantuck, Allan J., University of California, Los Angeles, Los Angeles, California; Dreicer, Robert, Cleveland Clinic Foundation, Cleveland, Ohio; Conlon, Kevin, Rush-Presbyterian-St. Luke's Medical Center, Chicago, Illinois; Sahasrabudhe, Deepak, University of Rochester Medical Center, Rochester, New York; Higano, Celestia, Seattle Cancer Care Alliance, Seattle, Washington; Ahmann, Frederick, University of Arizona Cancer Center, Tucson, Arizona; and Stadler, Walter, University of Chicago, Chicago, Illinois; *Randomized Multicenter Phase II Study Evaluating Two Dosing Schedules of TG4010 (MVA-MUC1-IL2) in Patients with Adenocarcinoma of the Prostate*. Sponsor: Transgene, Inc.

NIH/OBA Receipt Date: 9-12-01. Not Selected for RAC Public Review: 10-9-01

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**0109-498 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfusion/Human Telomerase Reverse Transcriptase (hTERT)/Intradermal Injections**

Vieweg, Johannes, Duke University Medical Center, Durham, North Carolina; *Phase I Study of Active Immunotherapy of Metastatic, Hormone Refractory Prostate Carcinoma using Autologous Mature Dendritic Cells (DC) Transfected with RNA Encoding Human Telomerase Reverse Transcriptase (hTERT)*.

NIH/OBA Receipt Date: 9-21-01. Not Selected for RAC Public Review: 10-26-01

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**0110-499 (Open) Gene Therapy/Phase I/Cancer/Liver (Hepatic) Metastases of Colorectal Cancer/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratatumoral Injection**

Sung, Max W., Mount Sinai Medical Center, New York, New York; *Clinical Trial of Adenoviral Vector Delivery of the Herpes Thymidine Kinase (HSV-tk) Gene by Intratumoral Injection Followed by Intravenous Ganciclovir with Imaging of HSV-tk Gene Expression in Patients with Hepatic Metastases from Colorectal Cancer*.

NIH/OBA Receipt Date: 10-9-01. Not Selected for RAC Public Review: 10-30-01

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**0110-500 (Open) Gene Therapy/Phase I/Cancer/Bladder/Pro-Drug/Ganciclovir/In Vivo/Adenovirus/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection**

Lerner, Seth P., Baylor College of Medicine, Houston, Texas; *Phase I Trial of Adenoviral Mediated Suicide Gene Therapy with HSV-tk Followed by Intravenous Administration of Ganciclovir in Patients with Locally Advanced and Refractory Superficial Bladder Cancer.*

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

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**0110-501 (Open) Gene Marking/Osteogenesis Imperfecta/In Vitro/CD34+ Cells from Donor Bone Marrow/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Infusion**

Horwitz, Edwin M., St. Jude Children's Research Hospital, Memphis, Tennessee; *Treatment of Children with Severe Osteogenesis Imperfecta by Stem Cell Transplantation and Mesenchymal Cell Graft Augmentation (Pilot Study).*

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

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**0110-502 (Open) Gene Therapy/Phase II/Peripheral Artery Disease/Plasmid DNA/Fibroblast Growth Factor 1 cDNA/Intramuscular Injection**

Comerota, Anthony J., Jobst Vascular Center, Toledo, Ohio; Mendelsohn, Farrell O., Cardiology, P.C., Birmingham, Alabama; Garza, Luis., Central Arkansas Veterans Healthcare System, Little Rock, Arkansas; Goldman, Corey K., Watson Clinic LLP, Lakeland, Florida; Greenbaum, Adam, Henry Ford Hospital, Detroit, Michigan; Sequeira, Rafael, Jackson Memorial Hospital, Miami, Florida; Henry, Timothy, Minneapolis Heart Institute Foundation, Minneapolis, Minnesota; Miller, Julie, The Johns Hopkins University, Baltimore, Maryland; Gray, John, Durham VA Medical Center, Durham, North Carolina; Hermiller, James and Irwin, Randy, St. Vincent Hospital and Health Care Center, Indianapolis, Indiana; Moneta, Gregory, Oregon Health & Science University, Portland, Oregon; Laird, John, Washington Hospital Center, Washington, DC; Chronos, Nicolas, Atlanta Cardiology Group, Atlanta, Georgia; Kent, K. Craig, Weill Medical College of Cornell University, New York, New York; Grossman, P. Michael, The University of Michigan Health Systems, Ann Arbor, Michigan; Eslami, Mohammad, Temple University, Philadelphia, Pennsylvania; Azrin, Michael, University of Connecticut Health Center, Farmington, Connecticut; Goldman, Corey, Ochsner Clinic Foundation, New Orleans, Louisiana; Rocha-Singh, Krishna, Prairie Heart Institute, Springfield, Illinois; and Lowman, Bruce G., Watson Clinic LLP, Lakeland, Florida; *A Phase II, Randomized, Double-Blind, Placebo Controlled, Parallel Group, Efficacy and Safety Study of Different Doses and Schedules of Administration of NV1FGF in Patients with Severe Peripheral Artery Occlusive Disease.* Sponsor: Aventis Pharma.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 11-15-01

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**0110-503 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Polylysine Polyethylene Glycol Complex/Intranasal Administration**

Konstan, Michael W., Case Western Reserve University, Cleveland, Ohio; and Wagener, Jeffrey, University of Colorado School of Medicine, Denver, Colorado; *Single Dose Escalation Study to Evaluate Safety of Nasal Administration of CFTR001 Gene Transfer Vector to Subjects with Cystic Fibrosis.*

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01  
Completed: 11-19-03

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**0110-504 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor  $\alpha$  and  $\beta$  Chain cDNA/Intravenous Infusion**

McDonagh, Kevin T., The University of Michigan Health System, Ann Arbor, Michigan; *A Phase I Study of Genetically Modified Autologous Peripheral Blood T-Cells Expressing a Retrovirally Encoded, MART-1 Specific  $\alpha\beta$  T-Cell Receptor, With and Without Recombinant Human Interleukin-2, in HLA-A2+.*

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

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**0110-505 (Closed) Gene Therapy/Phase I/Cancer/MUC1 Expressing Carcinoma/Immunotherapy/In Vivo/Naked Plasmid DNA/MUC-1/Intramuscular Injection**

Avigan, David E., Beth Israel Deaconess Medical Center, Boston, Massachusetts; *A Phase I Open-Label Study to Assess the Safety and Toxicity of Plasmid DNA MUC1 Vaccine (pMC6.5) in Metastatic Carcinoma.* Sponsor: Centocor, Inc.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 11-12-01  
Study never initiated: 4-18-03

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**0110-506 (Open) Gene Therapy/Phase I/Cancer/Melanoma/In Vitro/autologous T-Lymphocytes/Immunotherapy/Retrovirus/Interleukin-2 cDNA/Intravenous or Intra-arterial Infusion**

Rosenberg, Steven A., National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma using Lymphocytes Transduced with an Interleukin-2 (IL-2) Gene Following the Administration of a Nonmyeloablative but Lymphocyte Depleting Regimen.*

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 11-14-01

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**0110-507 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal Injection**

Wolchok, Jedd D., Memorial Sloan-Kettering Cancer Center, New York, New York; *Vaccination of AJCC Stage IIB, IIC, III and IV Melanoma Patients with a Multi-Epitope Peptide Vaccine using GM-CSF DNA as an Adjuvant: A Pilot Trial to Assess Safety and Immunity.*

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

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**0110-508 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/Antisense TAR/Antisense tat/rev/In Vitro/CD34+ Cells/Intravenous**

Krishnan, Amrita, City of Hope National Medical Center, Duarte, California; *Evaluation of the Safety and Efficacy of ex vivo Modification and Re-Infusion of CD34+ Cells by an Antisense Construct against HIV-1 in a Retrovirus Vector.* Sponsor: Enzo Therapeutics, Inc.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

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**0111-509 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Vector Directed Cell Lysis/In Vivo/Adenovirus/Serotype 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen Gene/Intratumoral Injection**

Corman, John M., Virginia Mason Medical Center, Seattle Washington; *A Phase I/II Trial of Intraprostatic Injection of CG7060 Followed by Three-Dimensional Conformal Radiation Therapy (3D-CRT) in Patients with Clinically Localized Intermediate or High-Risk Prostate Cancer.* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 11-9-01. Not Selected for RAC Public Review: 12-03-01  
Study was never initiated

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**0111-510 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Prostate Specific Antigen/Intradermal Injection**

Vieweg, Johannes, Duke University Medical Center, Durham, North Carolina; *Pilot Study evaluating the Migratory Patterns of Immature and In Vitro Matured Dendritic Cells Transfected with RNA Encoding PSA in Patients with Metastatic Prostate Cancer.*

NIH/OBA Receipt Date: 11-20-01. Not Selected for RAC Public Review: 12-11-01  
Closed: 2-02-04

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**0112-511 (Open) Gene Therapy/Phase I/Cancer Squamous Cell Carcinoma of the Head and Neck (SCCHN)/Immunotherapy/In Vitro/Allogeneic Tumor Cell/Retrovirus/Interleukin-2 cDNA/Intradermal Injection**

Johnson, Jonas T., University of Pittsburgh, Pittsburgh, Pennsylvania; *Active Immunization of Patients with Carcinoma of Oral Cavity or Oropharynx with Interleukin-2-Secreting Semi-allogeneic Human Carcinoma Cell Line Transfected with DNA from Autologous Tumor (Phase I Study).*

NIH/OBA Receipt Date: 12-20-01. Not Selected for RAC Public Review: 1-14-02

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**0112-512 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Cachexia/In Vivo/Plasmid/Chimeric Transactivator of Progesterone Receptor-Ligand-Binding Domain Fused to the Gal4 DNA Binding Domain/Human Growth Hormone Releasing Hormone (GHRH) cDNA/Intramuscular Injection**

Popat, Uday, Baylor College of Medicine, Houston, Texas; *Phase I Study of Human Growth Hormone Releasing Hormone Expressed by a Plasmid DNA Myogenic Vector in Patients with Cancer Cachexia.*

NIH/OBA Receipt Date: 12-21-01. Publicly Reviewed at the March 2002 RAC meeting

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**0201-513 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer (NSCLC)/In Vivo/Tumor Suppressor Gene/Cationic Liposome Complex/DOTAP:Cholesterol/Fus 1 cDNA/Intravenous Injection**

Lu, Charles, The University of Texas, M.D. Anderson Cancer Center, Houston, Texas; *Phase I Study of Intravenous DOTAP:Cholesterol-Fus 1 Liposome Complex (DOTAP:Chol-Fus 1) in Patients with Advanced Non-Small Cell Lung Cancer (NSCLC) Previously Treated with Chemotherapy.*

NIH/OBA Receipt Date: 01-08-02. Publicly Reviewed at the March 2002 RAC meeting

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**0201-514 (Open; RAC Reviewed with Recommendations) Gene Marking/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Serotype 2/Human Placental Alkaline Phosphatase (AP or hpAP) cDNA/Nasal and Bronchial Administration**

Aitken, Moira L., and Miller, A. Dusty, University of Washington, Seattle, Washington; *Transduction of the Upper and Lower Airway Epithelium in Healthy Subjects by an AAV2 Vector that Encodes Human Placental Alkaline Phosphatase.*

NIH/OBA Receipt Date: 01-09-02. Publicly Reviewed at the March 2002 RAC meeting

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**0201-515 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/In Vivo/Herpes Simplex Virus Type 1/HSV Thymidine Kinase (TK), Connexin 43, Tumor Necrosis Factor Alpha (TNF- $\alpha$ ), and the Viral Infected Cell Protein Zero (ICP0) Genes/Ganciclovir/Intratumoral (Stereotactic) Injections**

Lunsford, L. Dade, and Glorioso, Joseph C., University of Pittsburgh, School of Medicine, Pittsburgh, Pennsylvania; *Gene Therapy of Progressive Glioblastoma Multiforme using a Replication Defective HSV Multigene Vector NUREL-C2: A Phase I Clinical Trial to Determine the Maximum Tolerable Dose of Vector in Combination with Ganciclovir and Radiosurgery.*

NIH/OBA Receipt Date: 01-09-02. Publicly Reviewed at the March 2002 RAC meeting

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**0201-516 (Open) Gene Therapy/Phase I-II/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/In Vitro/Autologous CD34+ Cells from Peripheral Blood/Retrovirus  $\gamma$ cDNA/Intravenous Infusion**

Malech, Harry L., National Institutes of Health, Bethesda, Maryland; *Ex Vivo Retroviral Gene Transfer for Treatment of X-Linked Severe Combined Immunodeficiency (XSCID).*

NIH/OBA Receipt Date: 1-15-02. Not Selected for RAC Public Review: 2-5-02

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**0203-517 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Interferon-beta cDNA/Intratumoral Injection**

Dinney, Colin P., University of Texas M.D. Anderson Cancer Center; *An Open Label, Dose-Escalation Study to Determine the Tolerability of Interferon-beta (BG00001) Gene Transfer in the Neoadjuvant Treatment of High-Risk Resectable Prostate Cancer.* Sponsor: Biogen Idec, Inc.

NIH/OBA Receipt Date: 3-5-02. Not Selected for RAC Public Review: 3-25-02

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**0203-518 (Submission Not Complete) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Fowlpox Virus/Prostate Specific Antigen/Intradermal Injection**

*Phase II Randomized Study of Fowlpox PSA Vaccine with and without GM-CSF in the Treatment of Advanced Prostate Cancer.* Sponsor: Eastern Cooperative Oncology Group

NIH/OBA Receipt Date: 3-11-02.

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**0203-519 (Closed) Gene Therapy/Phase II/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection**

Laheru, Daniel, John Hopkins University School of Medicine, Baltimore, Maryland; and Nemunaitis, John J., Mary Crowley Medical Research Center, Dallas, Texas; *A Phase II Trial of CG8020 and CG2505 in Patients with Nonresectable or Metastatic Pancreatic Cancer.* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 3-15-02. Not Selected for RAC Public Review: 4-4-02  
Closed to accrual: 3-13-02

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**0203-520 (Open) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/Pro-Drug/Ganciclovir/In Vitro/Allogeneic T Lymphocytes/Retrovirus/Herpes Simplex Thymidine Kinase cDNA/Graft-Versus-Host Disease/Intravenous Infusion**

Orchard, Paul J., University of Minnesota Medical School, Minneapolis, Minnesota; *Transplantation of Unrelated or Mismatched Related Donor T Cells Containing the HSV-TK Suicide Gene to Facilitate Engraftment and Control Graft-Versus-Host Disease in Patients with Fanconi Anemia. A Phase I Trial.*

NIH/OBA Receipt Date: 3-21-02. Not Selected for RAC Public Review: 6-21-02

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**0204-521 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Peripheral Artery Disease/End Stage Renal Disease/Stenosis Prevention/In Vivo/Adenovirus/Inducible Nitric Oxide Synthase (iNOS) cDNA/Administration at the Arteriovenous (AV) Graft for Hemodialysis Access**

Tzeng, Elizabeth, University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *Inducible Nitric Oxide Synthase Gene Therapy for the Prevention of Intimal Hyperplasia in Arteriovenous Grafts used for Hemodialysis Access.*

NIH/OBA Receipt Date: 4-1-02. Publicly Reviewed at the June 2002 RAC meeting

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**0204-522 (Open) Gene Therapy/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen cDNA/B7.1 (CD80) cDNA/Intramuscular or Intradermal Injection**

Dahut, William, National Institutes of Health, Bethesda, Maryland; *A Pilot Trial of Concurrent Docetaxel and Pox Vector PSA Vaccine Followed by Docetaxel in Metastatic Androgen Independent Prostate Cancer.*

NIH/OBA Receipt Date: 4-9-02. Not Selected for RAC Public Review: 4-29-02

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**0204-523 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vivo/Measles Virus/Carcinoembryonic Antigen (CEA) cDNA/Intraperitoneal Administration**

Galanis, Evanthia, Mayo Clinic, Rochester, Minnesota; *Phase I Trial of Intraperitoneal Administration of a CEA Expressing Derivative Manufactured from a Genetically Engineered Strain of Measles Virus in Patients with Recurrent Ovarian Cancer.*

NIH/OBA Receipt Date: 4-11-02. Publicly Reviewed at the June 2002 RAC meeting

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**0204-524 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Intramuscular or Intradermal Injection**

Arlen, Philip M., National Institutes of Health, Bethesda, Maryland; *A Pilot Study of Sequential Vaccinations with Recombinant Vaccinia-CEA(6D)-TRICOM, and Recombinant Fowlpox-CEA(6D)-TRICOM (B7.1/ICAM-1/LFA-3) with Sargramostim (GM-CSF), in Conjunction with Standard Adjuvant Chemotherapy in High Risk Breast Cancer Patients Status Post Surgery with 4+ or More Lymph Nodes and CEA Expressing Tumors*

NIH/OBA Receipt Date: 4-16-02. Not Selected for RAC Public Review: 5-6-02

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**0204-525 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal Administration**

Wolf, Judith K., The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Dose Escalation Study of Intraperitoneal tgDCC-E1A and Intravenous Carboplatin for Treatment of Recurrent, Platinum-Sensitive Ovarian Cancer.* Sponsor: Targeted Genetics Corp.

NIH/OBA Receipt Date: 4-24-02. Not Selected for RAC Public Review: 5-14-02

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**0204-526 (Open) Gene Therapy/Phase I/Cancer/Colon Carcinoma (Hepatic Metastasis)/Herpes Simplex Virus Type-1/Tumor Lysis/Intrahepatic Artery Administration**

Fong, Yuman, Memorial Sloan-Kettering Cancer Center, New York, New York; *A Phase I, Open-Label, Dose-Escalating Study of Safety, Tolerability, and Anti-Tumor Activity of a Single Intrahepatic Arterial Injection of a Genetically Engineered Herpes Simplex Virus, NV1020, in Herpes Simplex Seronegative Subjects with Adenocarcinoma of the Colon with Metastasis to the Liver.* Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 4-23-02. Not Selected for RAC Public Review: 5-13-02

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**0204-527 (Open) Gene Therapy/Phase I/Cancer/Colon Carcinoma (Hepatic Metastasis)/Herpes Simplex Virus Type-1/Tumor Lysis/Intrahepatic Artery Administration**

Fong, Yuman, Memorial Sloan-Kettering Cancer Center, New York, New York; *Long-Term Follow-Up of the Safety and Survival of HSV Simplex Seronegative Subjects with Adenocarcinoma of the Colon with Metastasis to the Liver Who Enrolled in a Phase I Dose-Escalating Study Evaluating Genetically Engineered Herpes Simplex Virus, NV1020*. Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 4-23-02. Not Selected for RAC Public Review: 5-13-02

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**0204-528 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Disorders/Erectile Dysfunction/In Vivo/Plasmid/Human Maxi-K Channel hSlo cDNA/Intracavernous Injection**

Bar-Chama, Natan; Mt. Sinai School of Medicine., New York, New York; *Pilot Study of the Human hSlo/Maxi-K Gene to Treat Erectile Dysfunction*.

NIH/OBA Receipt Date: 4-23-02. Publicly Reviewed at the June 2002 RAC meeting

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**0204-529 (Open; RAC Reviewed with Recommendations) Gene Therapy/Other Disorders/Intractable Pain/In Vivo/Herpes Simplex Virus Type 1/Proenkephalin/Subcutaneous Inoculation**

Fink, David, and Glorioso, Joseph, University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *Gene Transfer for Intractable Pain: A Phase I Clinical Trial to Determine the Maximum Tolerable Dose of a Replication Defective HSV Vector Expressing Human Proenkephalin*.

NIH/OBA Receipt Date: 4-24-02. Publicly Reviewed at the June 2002 RAC meeting

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**0204-530 (Open) Gene Therapy/Phase II/Cancer/Pancreatic Cancer/Immunotherapy/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection**

Senzer, Neil Nathan, U.S. Oncology, Inc., Dallas Texas; Richards, Donald, Tyler Care Center, Tyler, Texas; Hecht, J. Randolph, UCLA Medical Center, Los Angeles, California; Hanna, Nader, University of Kentucky, Lexington, Kentucky; Chung, Theodore D.K., Virginia Commonwealth University, Richmond, Virginia; Vogel, Stephen, The University of Florida, Gainesville, Florida; Reid, Tony, University of California, San Diego Cancer Center, La Jolla, California; Chang, Kenneth J., University of California, Irvine, Orange, California; Javle, Milind, Roswell Park Cancer Institute, Buffalo, New York; Erickson, Richard, Scott & White Memorial Hospital and Clinic, Temple, Texas; Rosemurgy, Alexander, University of South Florida, Tampa, Florida; Posner, Mitchell C., University of Chicago, Chicago, Illinois; Perry, David, Washington Cancer Institute, Washington, D.C. Kallab, Andre, Medical College of Georgia, Augusta, Georgia; Sharma, Anand K., Medical University of South Carolina; Charleston, South Carolina; Chak, Amitabh, Case Western University Hospitals, Cleveland, Ohio; Harris, Hobart, San Francisco General Hospital, San Francisco, California; and Wadler, Scott; Weill Medical College, Cornell University, New York, New York; *A Randomized, Phase II, Study of TNFerade™ Biologic with 5-FU and Radiation Therapy for First-Line Treatment of Unresectable Locally Advanced Pancreatic Cancer*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 4-24-02. Not Selected for RAC Public Review: 5-14-02

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**0204-531 (Open) Gene Therapy/Cancer/Mesothelioma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interferon-beta/Intrapleural Administration**

Sterman, Daniel, University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Phase I Trial of Intrapleural Gene Therapy of Malignant Pleural Disease Using E1-Deleted Adenoviruses Containing the Human Interferon Beta Gene*.

NIH/OBA Receipt Date: 4-24-02. Not Selected for RAC Public Review: 5-14-02

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**0204-532 (Closed) Gene Therapy/Other Disorders/Peripheral Arterial Occlusive Disease (PAOD)/In Vivo/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intramuscular Injection**

Haser, Paul B., St. Michael's Medical Center, Newark, New Jersey; *Double-Blind, Randomized, Placebo-Controlled Study of Ascending Doses on Tolerability of Ad5.1 Mediated Human FGF-4 Gene Transfer Given Intramuscularly on One Day in Patients with Peripheral Arterial Occlusive Disease (PAOD) Fontaine Stage III or Fontaine Stage IV*. Sponsor: Berlex Laboratories.

NIH/OBA Receipt Date: 4-24-02. Not Selected for RAC Public Review: 7-10-02  
Closed: 12-16-02

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**0204-533 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Prostate/Radiotherapy/In Vivo/Adenovirus/Serotype 5/Human Sodium-Iodide Symporter (NIS) cDNA/Intratumoral Injection**

Morris, John C., Mayo Clinic, Rochester, Minnesota; *Phase I Trial of In Situ Gene Therapy for Locally Recurrent Prostate Cancer Following Radiation Therapy Failure Using Sodium/Iodide Symporter and Radioiodine.*

NIH/OBA Receipt Date: 4-25-02. Publicly Reviewed at the June 2002 RAC meeting

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**0204-534 (Open; RAC Reviewed with Recommendations) Non-therapeutic (Healthy Volunteers)/Phase I/In Vivo/Adenovirus/Serotype 4/Human Immunodeficiency Virus-1 env Plus rev or gag/Protease Plus rev Inserted Genes/Oral or Intranasal Administration**

Connors, Mark, National Institutes of Health, Bethesda, Maryland; *Phase I Study of AD4- $\Delta$ E3-HIV<sub>env</sub> and AD4- $\Delta$ E3-HIV<sub>gag/pro</sub> Recombinant Vaccines in HIV-negative Volunteers.*

NIH/OBA Receipt Date: 4-24-02. Publicly Reviewed at the June 2002 RAC meeting

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**0205-535 (Open) Gene Therapy/Phase I/Cancer/Acute Lymphoblastic Leukemia (ALL)/Immunotherapy/In Vitro/Plasmid DNA/Chimeric T Cell Receptor (CD19R) cDNA/Fusion Gene Encoding Hygromycin Phosphotransferase and Herpes Simplex Thymidine Kinase (HyTK)/Intravenous Infusion**

Cooper, Laurence J. N., City of Hope Medical Center, Duarte, California; *Phase I Study to Evaluate the Safety of Cellular Immunotherapy for High-Risk CD19+ Acute Lymphoblastic Leukemia after Autologous Hematopoietic Stem Cell Transplantation using Genetically Modified CD19-redirected Autologous Cytolytic T Cell Clones.*

NIH/OBA Receipt Date: 5-6-02. Not Selected for RAC Public Review: 5-28-02

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**0205-536 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection**

Kaufman, Howard L., Columbia University, New York, New York; Plante, Mark, The University of Vermont, Burlington, Vermont; DiPaola, Robert, Robert Wood Johnson Medical School, New Brunswick, New Jersey; and Israeli, Ronald, Staten Island Urological Research PC, Staten Island, New York; *Phase I Open Label Study to Evaluate the Safety of PROSTVAC-VF-TRICOM in the Treatment of Subjects with Adenocarcinoma of the Prostate.* Sponsor: Therion Biologics Corporation.

NIH/OBA Receipt Date: 5-16-02. Not Selected for RAC Public Review: 6-26-02

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**0205-537 (Open) Gene Therapy/Phase I-II/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Intramuscular Or Intradermal Injection**

Kasten-Sportes, Claude, National Institutes of Health, Bethesda, Maryland; *A Phase I-II Study of Tumor Antigen (CEA) Immunization with Autologous Peripheral Progenitor Cell Transplantation in Patients Previously Untreated for Metastatic Breast Cancer.*

NIH/OBA Receipt Date: 5-24-02. Not Selected for RAC Public Review: 6-14-02

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**0205-538 (Open) Gene Therapy/Phase I-II/Cancer/Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/p53 cDNA/Intradermal Injection**

Antonia, Scott J., University of South Florida, Tampa, Florida; *A Phase I-II Trial Using Dendritic Cells Transduced with an Adenoviral Vector Containing the p53 Gene to Immunize Patients with Extensive Stage Small Cell Lung Cancer after Standard Chemotherapy.*

NIH/OBA Receipt Date: 5-24-02. Not Selected for RAC Public Review: 6-14-02

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**0206-539 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Other Disorders/Superficial Corneal Opacity/Corneal Scarring/InVivo/Retrovirus/dnG1 Cyclin/Eye Administration (Ophthalmic Instillation)**

Song, Jonathan C., Keck School of Medicine, University of Southern California, Los Angeles, California; *Phase I/II Evaluation of Safety and Efficacy of a Matrix-Targeted Retroviral Vector Bearing a Dominant Negative Cyclin G1 Construct (Mx-dnG1) as Adjunctive Intervention for Superficial Corneal Opacity/Corneal Scarring.*

NIH/OBA Receipt Date: 6-5-02. Publicly Reviewed at the September 2002 RAC meeting

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**0206-540 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal Administration**

Wolf, Judith K., The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Dose Escalation Study of Intraperitoneal tgDCC-E1A and Intravenous Paclitaxel in Women with Platinum-Resistant Ovarian Cancer*. Sponsor: Targeted Genetics Corp.

NIH/OBA Receipt Date: 6-6-02. Not Selected for RAC Public Review: 6-26-02

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**0206-541 (Open) Gene Therapy/Phase I-II/Cancer/Breast/Immunotherapy/In Vivo/Naked Plasmid/Gene Encoding NY-ESO-1 Epitope/Intralymphnodal Injection**

Waisman, James R., University of Southern California, Los Angeles, California; *A Phase I/II Study of Intranodal Delivery of Synchronovax BPL Vaccine, an Epitope Synchronization Plasmid DNA Vaccine, in Stage IV Breast Carcinoma Patients*. Sponsor: CTL ImmunoTherapies Corporation.

NIH/OBA Receipt Date: 6-13-02. Not Selected for RAC Public Review: 7-18-02

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**0207-542 (Open) Gene Therapy/Phase I-II/Cancer/Pancreas/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Intratatumoral Injection**

Fernandez-del Castillo, Carlo, Massachusetts General Hospital, Boston, Massachusetts; Aguilar-Cordova, Estuardo, Harvard Gene Therapy Initiative, Boston, Massachusetts; and Fisher, William, Baylor College of Medicine, Houston, Texas; *AdV-tk Gene Therapy in Combination with Chemoradiation for Locally Advanced Pancreatic Cancer*.

NIH/OBA Receipt Date: 7-17-02. Not Selected for RAC Public Review: 8-6-02

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**0207-543 (Open) Gene Therapy/Phase I/Cancer/Follicular Lymphoma/Immunotherapy/In Vitro/Plasmid DNA/Chimeric T Cell Receptor (CD19R) cDNA/Fusion Gene Encoding Hygromycin Phosphotransferase and Herpes Simplex Virus Thymidine Kinase**

Cooper, Laurence J. N., City of Hope National Medical Center, Duarte, California; *Phase I Study to Evaluate the Safety of Cellular Immunotherapy for CD 19+ Follicular Lymphoma Using Autologous T Cell Cytolytic Clones Genetically Modified to be CD19-Specific and Express HyTK*.

NIH/OBA Receipt Date: 7-18-02. Not Selected for RAC Public Review: 8-7-02

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**0207-544 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Melanoma/Pro-Drug/Valacyclovir/In Vivo/DNA-Liposome Complexes/Herpes Simplex Thymidine Kinase cDNA/Intravenous Injection**

Thompson, John A., Seattle Cancer Care Alliance and The University of Washington, Seattle, Washington; *A Phase I Study to Evaluate the Safety and Pharmacokinetics of Pro-1, a Liposome-Encapsulated Thymidine Kinase Gene Formulation, in Patients with Stage IV Metastatic Melanoma*. Sponsor: Protiva Biotherapeutics, Inc.

NIH/OBA Receipt Date: 7-24-02. Publicly Reviewed at the September 2002 RAC meeting

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**0207-545 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Peripheral Blood Lymphocytes/Plasmid DNA/Immunoglobulin Heavy (H) Chain Gene/Telomerase Reverse Transcriptase (hTERT) Gene/Intravenous Infusion**

Zanetti, Maurizio, University of California, San Diego, San Diego, California; *A Phase I, Escalating Dose, Open Label Evaluation of Safety, Feasibility and Tolerability of Transgenic Lymphocyte Immunization Vaccine (TLI) in Subjects with Histologically Proven Prostate Adenocarcinoma*. Sponsor: Cosmo Bioscience, Inc.

NIH/OBA Receipt Date: 7-24-02. Publicly Reviewed at the September 2002 RAC meeting

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**0207-546 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Peripheral Artery Disease/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection**

Powell, Richard J., Dartmouth Medical School, Lebanon, New Hampshire; Daniel, George K., The Care Group, LLC, Indianapolis, Indiana; Davies, Mark G., University of Rochester Medical Center, Rochester, New York; Comerota, Anthony J., Jobst Vascular Center, Toledo, Ohio; Henry, Timothy D., Minneapolis Heart Institute Foundation; Rockson, Stanley, Stanford University School of Medicine, Stanford, California; Young, John J., Linder Clinical Trial Center, Cincinnati, Ohio; Mendelsohn, Farrell, Cardiology, P.C., Birmingham, Alabama; Lopez, John, The University of Chicago, Chicago, Illinois; Chronos, Nicholas, American Cardiovascular Research Institute, Atlanta, Georgia; Conte, Michael, Brigham and Women's Hospital, Boston, Massachusetts; Cooper, Christopher, Medical College of Ohio, Toledo, Ohio; Faries, Peter, New York Presbyterian Hospital, Weill Medical College at Cornell University, New York, New York; Goldman, Corey, Oschner Clinic Foundation, New Orleans, Louisiana; Kwolek, Christopher, Massachusetts General Hospital, Boston, Massachusetts; Saucedo, Jorge, Oklahoma Medical Center, Oklahoma City, Oklahoma; Farber, Alik, Midway Hospital Medical Center, Los Angeles, California; Bandyk, Dennis, University of South Florida, Tampa, Florida; Moursi, Mohammed, Central Arkansas Veterans Healthcare System, Little Rock, Arkansas; and Sidawy, Anton; Veteran Affairs Medical Center, Washington, DC; *A Phase I/II Double-Blind, Randomized, Placebo-Controlled Study to Assess the Safety and Efficacy of AMG0001 to Improve Perfusion in Critical Leg Ischemia*. Sponsor: AnGes, Inc.

NIH/OBA Receipt Date: 7-24-02. Publicly Reviewed at the September 2002 RAC meeting

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**0207-547 (Withdrawn from RAC Review) Gene Therapy/Phase I-II/Peripheral Artery Disease/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection**

Powell, Richard J., Dartmouth Medical School, Lebanon, New Hampshire; *A Phase I/II Double-Blind, Randomized, Placebo-Controlled Study to Assess the Safety and Efficacy of AMG0001 to Improve Perfusion and Healing After Major Amputation Due to Critical Leg Ischemia*. Sponsor: AnGes, Inc.

NIH/OBA Receipt Date: 7-24-02. Withdrawn from RAC review: 9-6-02.

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**0207-548 (Open) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous Infusion**

Vieweg, Johannes and Chao, Nelson, Duke University Medical Center, Durham, North Carolina; *Active Immunotherapy with Mature, Tumor RNA-Transfected, Autologous Dendritic Cells with or without the IL2-Diphtheria Toxin Conjugate Denileukin Diftox (Ontak®) in Patients with Metastatic Renal Cell Carcinoma*.

NIH/OBA Receipt Date: 7-29-02. Not Selected for RAC Public Review: 8-16-02

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**0208-549 (Open) Gene Therapy/Phase II/Cancer/Esophagus/Immunotherapy/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection**

Senzer, Neil, US Oncology, Dallas, Texas; Swisher, Stephen G., M.D. Anderson Cancer Center, Houston, Texas; Reid, Tony, University of California, San Diego Cancer Center, La Jolla California; Hanna, Nader, University of Kentucky, Lexington, Kentucky; Shabahang, Mohammad-Mohsen, Scott and White Memorial Hospital and Clinic, Temple, Texas; Mauer, Ann, University of Chicago Cancer Research Center, Chicago, Illinois; Chung, Ted, Massey Cancer Center, Virginia Commonwealth University, Richmond, Virginia; and Rosemurgy, Alexander, University of South Florida, Tampa, Florida; Chang, Kenneth J., University of California, Irvine, Orange, California; Kleinberg, Lawrence, Sidney Kimmel Cancer Center, Johns Hopkins, Baltimore, Maryland; and Chak, Amitabh; Case Western University Hospitals, Cleveland, Ohio; *A Phase II, Multi-Center, Single Arm Evaluation of Preoperative Chemoradiation Plus TNFerade™ Biologic (Ad<sub>GV</sub>EGR.TNF.11D) Prior to Esophagectomy for Locally Advanced Esophageal Cancer*. Sponsor: GenVec.

NIH/OBA Receipt Date: 8-1-02. Not Selected for RAC Public Review: 8-21-02

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**0208-550 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/ $\alpha$ -(1,3) galactosyltransferase Gene/Subcutaneous Injection**

Morton, Roscoe F., Iowa Methodist Medical Center, Des Moines, Iowa; *A Phase I/II Study of an Antitumor Vaccination Using  $\alpha$ (1,3) galactosyltransferase Expressing Allogeneic Tumor Cells in Patients with Relapsed or Refractory Breast Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 8-26-02. Publicly Reviewed at the December 2002 RAC meeting

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**0210-551 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/Vaccinia Virus/Tyrosinase cDNA/Intramuscular Injection**

Topalian, Suzanne, National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma using Recombinant Vaccinia and Fowlpox Viruses Encoding the Tyrosinase Antigen in Combination with Interleukin-2*.

NIH/OBA Receipt Date: 10-3-02. Not Selected for RAC Public Review: 10-24-02

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**0210-552 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/ $\alpha(1, 3)$ galactosyltransferase Gene/Subcutaneous Injection**

Morris, John C., National Institutes of Health, Bethesda, Maryland; *A Phase I/II Study of an Antitumor Vaccination using  $\alpha(1, 3)$ galactosyltransferase Expressing Allogeneic Tumor Cells in Patients with Refractory or Recurrent Non-Small Cell Lung Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 10-9-02. Publicly Reviewed at the December 2002 RAC meeting

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**0210-553 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic B-Leukemia/Immunotherapy/In Vitro/Plasmid DNA/Interleukin-2/CD40 Ligand/Subcutaneous Injections**

Brenner, Malcolm, Baylor College of Medicine, Houston, Texas; *Treatment of Chronic Lymphocytic B-Leukemia (B-CLL) with Human IL-2 and Human CD40 Ligand Plasmid Gene Modified Autologous Tumor Cells*.

NIH/OBA Receipt Date: 10-9-02. Not Selected for RAC Public Review: 10-30-02

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**0210-554 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Infectious Diseases/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag-Pol-Nef-Env cDNA/Interleukine-2 (IL-2)/Ig Fusion Protein/Bioinjector 2000® Injections**

Dolin, Raphael, Harvard Medical School, Boston Massachusetts; Blattner, William, University of Maryland, Baltimore, Maryland; and Hammer, Scott Columbia University/New York Blood Center, New York, New York; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of the HIV-1 DNA Vaccine VRC-HIVDNA009-00-VP (Gag-Pol-Nef-Multiclade Env) with the Plasmid Cytokine Adjuvant VRC-ADJDNA004-IL2-VP (IL-2/Ig)*.

NIH/OBA Receipt Date: 10-9-02. Not Selected for RAC Public Review: 10-30-02

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**0210-555 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid/Prostate Specific Antigen (PSA)/Intramuscular Injection**

Malkowicz, S. Bruce, University of Pennsylvania Health System, Philadelphia, Pennsylvania; *A Phase I Study of a Polynucleotide Anti-Tumor Immunization to Human Prostate Specific Antigen (PSA) in Patients with Hormone-Refractory Prostate Cancer (HRPC)*. Sponsor: Centocor Inc.

NIH/OBA Receipt Date: 10-9-02. Not Selected for RAC Public Review: 10-30-02

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**0210-556 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Plasmid DNA/Adenovirus/Serotype 5/L523S cDNA/Intramuscular Injection**

Nemunaitis, John J., US Oncology, Dallas, Texas; Anthony, Stephen P., Cancer Care Northwest, Spokane, Washington; Richards, Donald, Tyler Cancer Center, Tyler, Texas; Berman, Barry S., Cancer Centers of Florida, Ocoee, Florida; and West, Howard, Swedish Cancer Institute, Seattle, Washington; *Phase I Open-Label, Dose Escalation Trial Evaluating the Safety and Immunogenicity of Sequential Administration of Recombinant DNA and Adenovirus Expressing L523S Protein in Patients with Early Stage Non-Small Cell Lung Cancer*. Sponsor: Corixa Corporation

NIH/OBA Receipt Date: 10-9-02. Publicly Reviewed at the December 2002 RAC meeting

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**0210-557 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Coronary Artery Disease/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramyocardial Injection**

Simons, Michael, Dartmouth Medical School, Lebanon, New Hampshire; and Annex, Brian H., Duke University School of Medicine, Durham VA Medical Center, Durham, North Carolina; *A Double-Blind, Placebo-Controlled, Dose Escalation Pilot Study to Assess the Safety and Effects of AMG0001 in Patients with Ischemic Heart Disease (IHD) not Amenable to Coronary Artery Bypass Graft (CABG) or Percutaneous Coronary Intervention (PCI)*. Sponsor: AnGes, Inc.

NIH/OBA Receipt Date: 10-9-02. Publicly Reviewed at the December 2002 RAC meeting

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**0212-558 (Open) Gene Therapy/Phase I-II/Cancer/Breast/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Serotype 5/p53 cDNA/Subcutaneous Injection**

Reed, Elizabeth C., University of Nebraska Medical Center, Omaha, Nebraska; *Adenovirus p53 Infected DC Vaccine for Breast Cancer*.

NIH/OBA Receipt Date: 12-9-02. Not Selected for RAC Public Review: 1-13-03

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**0212-559 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection**

Kaufman, Howard L., Columbia University, New York, New York; and Safran, Howard, The Miriam Hospital, Providence, Rhode Island; *An Open Label Phase I Study to Evaluate the Safety and Tolerability of rV-CEA(6D)/TRICOM™ Admixed with rV-MUC-1 followed by rF-CE(6D)/TRICOM™ in Combination with GM-CSF in Subjects with Unresectable Adenocarcinoma of the Pancreas.* Sponsor: Therion Biologics Corporation.

NIH/OBA Receipt Date: 12-10-02. Not Selected for RAC Public Review: 1-06-03

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**0212-560 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/GM-CSF/Intramuscular Or Intradermal Injection**

Arlen, Philip M., National Institutes of Health, Bethesda, Maryland; *A Phase I/II Pilot Study of Sequential Vaccinations with rFOWLPOX-PSA (L155)-TRICOM (PROSTAVAC-F/TRICOM) Alone, or in Combination with rVACCINIA-PSA (L155)-TRICOM (PROSTAVAC-V/TRICOM) and the Role of GM-CSF, in Patients with Prostate Cancer.* Sponsor: Therion Biologics Corporation.

NIH/OBA Receipt Date: 12-10-02. Not Selected for RAC Public Review: 1-06-03

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**0212-561 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection**

Shuman, Marc, University of California, San Francisco Cancer Center, San Francisco, California; *Pancreatic GVAX® for Resected Adenocarcinoma of the Pancreas.*

NIH/OBA Receipt Date: 09-04-02. Not Selected for RAC Public Review: 1-03-03

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**0212-562 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vitro/Allogeneic K562 Cell/Combination With Untransduced Tumor Cells/Plasmid DNA/Electroporation/DMRIE-Cholesterol/Granulocyte-macrophage Colony Stimulating Factor cDNA/CD40 Ligand cDNA/Intradermal Injection**

Dessureault, Sophie, University of South Florida, Tampa, Florida; *A Phase I Trial Using a Universal GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) in the Formulation of Autologous Tumor Cell-Based Vaccines for Cancer Patients with Stage IV Disease.*

NIH/OBA Receipt Date: 12-18-02. Not Selected for RAC Public Review: 1-10-03

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**0212-563 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/GD-2 Specific scFvFc-Zeta T Cell Receptor/Intravenous Injections**

Russell, Heidi, and Brenner, Malcolm, Baylor College of Medicine, Houston, Texas; *Administration of Peripheral Blood T-Cells and EBV Specific CTLs Transduced to Express GD-2 Specific Chimeric T Cell Receptors to Patients with Neuroblastoma.*

NIH/OBA Receipt Date: 12-24-02. Publicly Reviewed at the March 2003 RAC meeting

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**0301-564 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer Adenocarcinoma Expressing Carcinoembryonic Antigen (CEA)/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/Anti-CEA-sFv-Zeta T Cell Receptor-CD28/Intravenous Infusion**

Junghans, Richard, Beth Israel Deaconess Medical Center, Harvard Medical School, Boston, Massachusetts; *Phase Ia/Ib Trial of 2<sup>nd</sup> Generation Designer T Cells in Adenocarcinoma.*

NIH/OBA Receipt Date: 1-06-03. Publicly Reviewed at the March 2003 RAC meeting

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**0301-565 (Closed) Gene Therapy/Phase I/Anaplastic Thyroid Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5 p53 cDNA/Intratumoral Injections**

Reid, William K., Vanderbilt-Ingram Oncology, Vanderbilt University Medical Center, Franklin, Tennessee; *Study to Evaluate the Overall Response and Safety of Biweekly Intratumoral Administration of RPR/INGN 201 in Anaplastic Thyroid Cancer.*

NIH/OBA Receipt Date: 1-06-03. Not Selected for RAC Public Review

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**0301-566 (Open) Gene Therapy/Phase I/Cancer/Hematologic Malignancy Following Allogeneic Bone Marrow Transplantation/Pro-drug/Elimination of Graft Versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/CD34-Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous**

DiPersio, John, Washington University School of Medicine, St. Louis, Missouri; *Infusion of Genetically Modified T Cells: Tracking and Toxicity.*

NIH/OBA Receipt Date: 1-07-03. Not Selected for RAC Public Review: 5-13-03

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**0301-567 (Open) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Percutaneous Cardiac Catheterization/Intra-myocardial Injection**

Losordo, Douglas W., St. Elizabeth's Medical Center, Tufts University School of Medicine, Boston Massachusetts; *A Multicenter, Randomized, Double-Blind, Dose Ranging Placebo-Controlled Study Evaluating Defined Doses of Percutaneously Delivered Via Boston Scientific Stiletto™ Endocardial Direct Injection Catheter System pVGI.1 (VEGF2) (Placebo, 20, 200, or 800 µg) in Patients with Class III or IV.* Sponsor: Coraetus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/OBA Receipt Date: 1-08-03. Not Selected for RAC Public Review: 1-29-03

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**0301-568 (Open) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/DNA-Liposome Complexes/Poloxamer 188/Del-1 cDNA/Intramuscular Injection**

Rajagopalan, Sanjay, University of Michigan, Ann Arbor, Michigan; Rocha-Singh, Krishna, Prairie Education and Research Cooperative, Springfield, Illinois; Kleiman, Neal S., Baylor College of Medicine, The Methodist Hospital, Houston, Texas; Litt, Marc, Jacksonville Heart Center, Jacksonville, Florida; Hermiller, James B., The Care Group, LLC, Indianapolis, Indiana; Henry, Tim, Abbott Northwestern Hospital/Minneapolis Heart Institute Foundation, Minneapolis, Minnesota; Snell, Jeffrey, Rush-Presbyterian St. Luke's Medical Center, Chicago, Illinois; Grossman, P. Michael, University of Michigan Health Systems, Ann Arbor, Michigan; Mendelsohn, Farrell O., Cardiology P.C., Birmingham, Alabama; Kandzari, David, Duke University Medical Center, Durham, North Carolina; Weiss, Robert, Andoscoggin Cardiology Associates, Auburn, Maine; Ramaiah, Venkatesh, Arizona Heart Institute, Phoenix, Arizona; Powell, Richard, Dartmouth-Hitchcock Medical Center, Lebanon, New Hampshire; Chronos, Nicholas, American Cardiovascular Research Institute, Atlanta, Georgia; Gottlieb, Daniel, Buriel, Washington; Piana, Robert, Vanderbilt University Medical Center, Nashville, Tennessee; Anderson, R. David, Sarasota Memorial Health Care System, Sarasota, Florida; Saucedo, Jorge, University of Oklahoma Health Sciences Center, Oklahoma City, Oklahoma; and Karlsberg, Ronald P., Access Clinical Trials/Cardiovascular Research Institute, Beverly Hills, California; *A Phase II Multi-Center, Double-Blind, Placebo-Controlled, Trial of VLTS-589 in Subjects with Intermittent Claudication Secondary to Peripheral Arterial Disease.* Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 1-08-03. Not Selected for RAC Public Review: 1-29-03

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**0301-569 (Open) Gene Therapy/Phase II/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) cDNA/Aerosol Administration**

Moss, Richard, Stanford University School of Medicine, Palo Alto, California; Spencer, L. Terry, University of Florida, Gainesville, Florida; Clancy, John P., University of Alabama at Birmingham, Birmingham, Alabama; Zeitlin, Pamela L., Johns Hopkins University, Baltimore, Maryland; Milla, Carlos E., University of Minnesota, Minneapolis, Minnesota; Colombo, John L., University of Nebraska Medical Center, Omaha, Nebraska; Accurso, Frank, The Children's Hospital Denver, Denver, Colorado; Dorkin, Henry L., Massachusetts General Hospital, Boston, Massachusetts; Waltz, David A., Children's Hospital Boston, Boston, Massachusetts; Pilewski, Joseph M., University of Pittsburgh, Pittsburgh, Pennsylvania; Pian, Mark; Children's Hospital and Health Center, San Diego, California; and Ferkol, Thomas; Washington University School of Medicine, St. Louis, Missouri; *A Multicenter, Double-Blind, Placebo-Controlled, Phase II Study of Aerosolized tgAAVCF for the Treatment of Cystic Fibrosis.* Sponsor: Targeted Genetics Corporation.

NIH/OBA Receipt Date: 1-08-03. Not Selected for RAC Public Review: 1-29-03

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**0301-570 (Open; RAC Reviewed with Recommendations) Non-therapeutic (Healthy Volunteers)/Cholera Vaccine/In Vivo/Vibrio cholerae/Oral Administration**

Tacket, Carol O., Center for Vaccine Development, University of Maryland, Baltimore, Maryland; *Use of in vivo Expression Technology to Identify Virulence Factors and Protective Antigens of Vibrio cholerae 01.*

NIH/OBA Receipt Date: 1-08-03. Publicly Reviewed at the March 2003 RAC meeting

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**0302-571 (Open) Gene Therapy/Phase II/Immunotherapy/Cancer/Non-Small Cell Lung Carcinoma (NSCLC)/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Ctokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intradermal Injections**

Ross, Helen J., Providence Portland Medical Center, Robert W. Franz Cancer Research Center, Earle A. Chiles Research Institute, Portland, Oregon; Anthony, Stephen P., Cancer Care Northwest, Spokane, Washington; Kelly, Karen, University of Colorado, Denver, Colorado; Sterman, Daniel, University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; Harper, Harry, Hackensack University Medical Center, Hackensack, New Jersey; Carbone, David, Vanderbilt University Medical Center, Nashville, Tennessee; Figlin, Robert, University of California, Los Angeles, School of Medicine, Los Angeles, California; Jablons, David, University of California, San Francisco Comprehensive Cancer Center, San Francisco, California; and Schiller, Joan H., University of Wisconsin Comprehensive Cancer Center, Madison, Wisconsin; *A Phase II Randomized Study of GM-CSF Gene-Modified Autologous Tumor Vaccine (CG8123) With and Without Low-Dose Cyclophosphamide in Advanced Stage Non-Small Cell Lung Cancer.* Sponsor: Cell Genesys.

NIH/OBA Receipt Date: 2-11-03. Not Selected for RAC Public Review: 3-04-03

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**0302-572 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Adenovirus Type 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intratatumoral Injection**

Corman, John, Virginia Mason Medical Center, Seattle, Washington; Nemunaitis, John J., Mary Crowley Medical Research Center, Dallas, Texas; Nieva, Jorge; Scripps Cancer Center, San Diego, California; and DeWeese, Theodore; Johns Hopkins University, Baltimore, Maryland; *A Phase I/II Trial of Intraprostatic Injection of CG7870 Followed by Three-Dimensional Conformal Radiation Therapy (3D-CRT) in Patients with Clinically-Localized Intermediate-Risk Prostate Cancer.* Sponsor: Cell Genesys.

NIH/OBA Receipt Date: 2-24-03. Not Selected for RAC Public Review: 3-14-03

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**0303-573 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Human and Mouse gp100 cDNAs/Intramuscular Injection**

Wolchok, Jedd D., Memorial Sloan Kettering Cancer Center, New York, New York; *Injection of AJCC Stage IIB, IIC, III and IV Melanoma Patients with Human and Mouse gp100 DNA: A Phase I Trial to Assess Safety and Immune Response.*

NIH/OBA Receipt Date: 3-24-03. Not Selected for RAC Public Review: 4-11-03

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**0303-574 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer (NSCLC)/Immunotherapy/In Vitro/AD#100, an Allogeneic Human Lung Adenocarcinoma Cell Line/Plasmid DNA/Human Heat Shock Protein gp96-Ig cDNA/Intradermal Injections**

Raez, Luis E., University of Miami School of Medicine, Miami, Florida; *Novel Tumor Vaccine gp96-Ig Fusion Protein in Advanced (Stage III), Relapsed or Metastatic (Stage IV) Non-Small Cell Lung Cancer (NSCLC) Patients Who Have Failed First Line Chemotherapy.*

NIH/OBA Receipt Date: 3-24-03. Not Selected for RAC Public Review: 4-11-03

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**0304-575 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Disorders/ Retinitis Pigmentosa/In Vitro/Plasmid/Encapsulated Cell-Based Drug Delivery Device/Human Ciliary Neurotrophic Factor (CNTF) cDNA/Intraocular Implantation (Via Sclerotomy)**

Sieving, Paul A., National Institutes of Health, Bethesda, Maryland; *A Phase I Study of NT-501, an Implant of Encapsulated Human NTC-201 Cells Releasing Ciliary Neurotrophic Factor (CNTF), in Patients with Retinitis Pigmentosa.* Sponsor: Neurotech USA.

NIH/OBA Receipt Date: 4-10-03. Publicly Reviewed at the June 2003 RAC meeting

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**0304-576 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/In Vivo/Plasmid DNA/Epidermal Growth Factor Receptor Antisense/Intratatumoral Injection**

Grandis, Jennifer Rubin, University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *Phase I Trial of Intratumoral EGFR Antisense DNA in Advanced Head and Neck Squamous Cell Carcinoma.*

NIH/OBA Receipt Date: 4-11-03. Not Selected for RAC Public Review: 5-01-03

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**0304-577 (Open) Gene Therapy/Cancer/Renal Cell Cancer/Immunotherapy/Vaccinia Virus/5T4 cDNA/Intramuscular Injection**

Kaufman, Howard, Columbia University College of Physicians and Surgeons, New York, New York; *A Preliminary Study of the Safety, Immunogenicity and Clinical Efficacy of TroVax Given in Conjunction with Interleukin 2 in the Treatment of Stage IV Renal Cell Cancer.* Sponsor: Oxford BioMedica, plc.

NIH/OBA Receipt Date: 4-23-03. Not Selected for RAC Public Review: 5-12-03

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**0304-578 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection**

Emens, Leisha A., The Sidney Kimmel Comprehensive Cancer Center, Johns Hopkins University, Baltimore, Maryland; *A Phase I Vaccine Safety and Chemotherapy Dose-Finding Trial of GM-CSF-Secreting Breast Cancer Vaccine Given in a Specifically Timed Sequence with Immunomodulatory Doses of Cyclophosphamide and Doxorubicin.*

NIH/OBA Receipt Date: 4-22-03. Not Selected for RAC Public Review: 5-12-03

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**0304-579 (Open) Gene Therapy/Phase I/Infectious Disease/Cytomegalovirus (CMV) Disease/In Vitro/EBV and CMV-Specific Cytotoxic T Lymphocytes/Adenovirus/CMV pp65 Gene/Intravenous**

Bollard, Catherine; Texas Children's Hospital and Center for Cell and Gene Therapy and Baylor College of Medicine; Houston, Texas; *Virus Specific Cytotoxic T-Lymphocytes for the Prophylaxis of CMV after Allogeneic Stem Cell Transplant: A Dose Finding Trial.*

NIH/OBA Receipt Date: 4-22-03. Not Selected for RAC Public Review: 5-12-03

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**0304-580 (Open) Gene Therapy/Phase II/Cancer/Melanoma/In Vivo/Tumor Suppressor/Adenovirus/Melanoma Differentiation Associated Protein-7 cDNA/Intratumoral Injection**

Kim, Kevin B., The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Phase II Study Examining the Biological Efficacy of Intratumoral INGN 241 (Ad-mda7) Administration in Patients with In Transit Melanoma.* Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 4-22-03. Not Selected for RAC Public Review: 5-12-03

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**0304-581 (Open) Gene Therapy/Phase I/Cancer/Bladder/Immunotherapy/In Vivo/Fowlpox Virus/B7.1 (CD80)/ICAM-1/LFA-3/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intravesical Administration**

Wojtowicz, Malgorzata E., The Cancer Institute of New Jersey, UMDNJ-Robert Wood Johnson Medical School, New Brunswick, New Jersey; *Phase I Study of Intravesical Recombinant Fowlpox-GM-CSF (rF-GM-CSF) and/or Recombinant Fowlpox-TRICOM (rF-TRICOM) in Patients with Muscle-Invasive Bladder Carcinoma.* Sponsor: Therion Biologics Corporation

NIH/OBA Receipt Date: 4-23-03. Not Selected for RAC Public Review: 5-13-03

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**0305-582 (Open) Gene Therapy/Phase I-II/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal Administration**

Wolf, Judith The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase 1/2 Randomized Study of Intraperitoneal tgDCC-E1A and Intravenous Paclitaxel in Women with Platinum-Resistant Ovarian Cancer.*

NIH/OBA Receipt Date: 5-30-03. Not Selected for RAC Public Review: 6-20-03

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**0306-583 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo Electroporation/Plasmid DNA/Interleukin-12 cDNA/Intratumoral In Vivo Electroporation**

Daud, Adil, H. Lee Moffitt Cancer Center, Tampa, Florida; *Phase I Trial of Intratumoral pIL-12 Electroporation in Malignant Melanoma.*

NIH/OBA Receipt Date: 6-19-03. Not Selected for RAC Public Review: 7-10-03

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**0307-584 (Open) Gene Therapy/Phase II/Immunotherapy/Cancer/Melanoma/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous and Intradermal Injection**

Hodi, F. Stephen, Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase II Trial of Vaccination with Autologous Lethally Irradiated Melanoma Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Granulocyte-Macrophage Colony Stimulating Factor in Stage III and IV Metastatic Melanoma Patients.*

NIH/OBA Receipt Date: 7-16-03. Not Selected for RAC Public Review: 8-05-03

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**0307-585 (Open) Gene Therapy/Phase I/Cancer/CEA Expressing Carcinomas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection**

Carson, William E., III, The Ohio State University, Columbus, Ohio; *A Phase I Study of Sequential Vaccinations with Fowlpox-CEA(6D)-TRICOM (B7.1/ICAM/LFA-3) and Vaccinia-CEA(6D)-TRICOM, in Combination with GM-CSF and Interferon-Alfa-2B in Patients with CEA Expressing Carcinomas.*

NIH/OBA Receipt Date: 7-22-03. Not Selected for RAC Public Review: 8-11-03

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**0307-586 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection**

Kaufman, Howard L., Columbia University, New York, New York; and Marshall, John, Georgetown University Medical Center, Washington, D.C.; *An Open Label Phase I Study to Evaluate the Safety and Tolerability of PANCAC™-VF in Combination with GM-CSF in Patients with Unresectable Adenocarcinoma of the Pancreas.*

NIH/OBA Receipt Date: 7-21-03. Not Selected for RAC Public Review: 8-08-03

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**0307-587 (Open) Gene Therapy/Phase II/Cancer/Colorectal/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection**

Libutti, Steven K., National Institutes of Health, Bethesda, Maryland; *A Phase II Randomized Trial Concerning TNFerade Biologic with Capecitabine and Radiation Therapy followed by Surgical Resection Versus Capecitabine and Radiation Therapy followed by Surgical Resection for the Treatment of Rectal Cancer.*

NIH/OBA Receipt Date: 7-21-03. Not Selected for RAC Public Review: 8-08-03

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**0307-588 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Rheumatoid Arthritis/In Vivo/Adeno-Associated Virus/Serotype 2/Tumor Necrosis Factor Receptor-Fc Immunoglobulin (TNFR:Fc) Fusion Gene cDNA/Intra-Articular Administration**

Mease, Philip J., Swedish Hospital Rheumatology Clinical Research Division, Seattle, Washington; Hobbs, Kathryn, Denver Arthritis Clinic, Denver, Colorado; and Bingham, Clifton; New York University School of Medicine, New York, New York; *A Phase I Dose Escalation Study of Intra-Articular Administration of tgAAC94, a Recombinant Adeno-Associated Vector Containing the TNFR:Fc Fusion Gene, in Rheumatoid Arthritis.* Sponsor: Targeted Genetics.

NIH/OBA Receipt Date: 7-26-03. Publicly Reviewed at the September 2003 RAC meeting

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**0307-589 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Glaucoma/In Vivo/Adenovirus/Serotype 5/p21<sup>WAF-1/Cip1</sup> cDNA/Subconjunctival Injection**

Kaufman, Paul L., University of Wisconsin-Madison Medical School, Madison, Wisconsin; and Weinreb, Robert N., University of California, San Diego, La Jolla, California; *A Phase 1 Study in Glaucoma Subjects Receiving SCH 412499 (rAd-p21) Administered as a Single Injection into the Subconjunctival Space Prior to Primary Trabeculectomy.* Sponsor: Schering Corporation.

NIH/OBA Receipt Date: 7-23-03. Publicly Reviewed at the December 2003 RAC meeting

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**0307-590 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/Yeast Cytosine Deaminase cDNA/5-Fluorocytosine/Herpes Simplex Thymidine Kinase cDNA/Valganciclovir/Adenovirus Death Protein/Intratumoral Injection**

Kim, Jae Ho, and Freytag, Svend, Henry Ford Health System, Detroit, Michigan; *Phase I/II Study of Replication-Competent Adenovirus-Mediated Double Suicide Gene Therapy in Combination with Conventional Dose Conformal Radiation Therapy for the Treatment of Intermediate to High-Risk Prostate Cancer*

NIH/OBA Receipt Date: 7-23-03. Not Selected for RAC Public Review: 8-12-03

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**0307-591 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Tumor Suppressor Gene/In Vivo/Cationic Liposome Complex/DOTAP-DOPE/p53 cDNA/Intravenous Infusion**

Marshall, John, Lombardi Cancer Center, Georgetown University Medical Center, Washington, DC; *An Open-Label Safety Study of Escalating Doses of SGT-53 for Systemic Injection in Patients with Advanced Solid Tumor Malignancies.*

NIH/OBA Receipt Date: 7-23-03. Not Selected for RAC Public Review: 8-12-03

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**0307-592 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Cervical Cancer/Immunotherapy/In Vivo/Listeria monocytogenes/Human Papilloma Virus E7 Gene/Intravenous Injection**

Marshall, John, Lombardi Cancer Center, Georgetown University Medical Center, Washington, DC; *A Phase 1 Study to Determine the Safety and Immunogenicity of Vaccination with Listeria monocytogenes Expressing Human Papilloma Virus Type 16 E7 for the Treatment of Progressive, Recurrent and Advanced Squamous Cell Cancer of the Cervix*. Sponsor: Advaxis, Inc.

NIH/OBA Receipt Date: 7-23-03. Publicly Reviewed at the December 2003 RAC meeting

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**0307-593 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Parkinson's Disease/In Vivo/Adeno-Associated Virus/Aromatic L-amino Acid Decarboxylase cDNA/Intrastriatal Administration**

Aminoff, Michael, University of California, San Francisco, San Francisco, California; *A Phase I Open-Label Safety Study of Intrastriatal Infusion of Adeno-Associated Virus Encoding Human Aromatic L-amino Acid Decarboxylase (AAV-hAAAADC-2) in Subjects with Advanced Parkinson's Disease*. Sponsor: Avigen.

NIH/OBA Receipt Date: 7-23-03. Publicly Reviewed at the October 17, 2003 RAC meeting

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**0307-594 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Degenerative Arthritis/In Vitro/Allogeneic Human Chondrocytes/Retrovirus/Transforming Growth Factor- $\beta$ 1 (TGF- $\beta$ 1)/Intra-Articular Administration**

Mont, Michael A., Sinai Hospital of Baltimore, Baltimore, Maryland; *A Phase 1 Study to Determine the Safety and Biological Activity of Cell-Mediated Gene Therapy Using TissueGene-C in Patients with Degenerative Joint Disease of the Knee Prior to Total Knee Arthroplasty*. Sponsor: TissueGene, Inc.

NIH/OBA Receipt Date: 7-23-03. Publicly Reviewed at the September 2003 RAC meeting

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**0307-595 (Open) Gene Therapy/Phase I-II/Cancer/Cervical/Immunotherapy/In Vivo/Plasmid DNA/HPV 16 E7 cDNA/Intramuscular Injection**

Trimble, Cornelia, Johns Hopkins University School of Medicine, Baltimore, Maryland; *A Phase I/II Clinical Trial of pNGVL4a-Sig/E7 (detox)/HSP70 for the Treatment of Patients with HPV16+ Cervical Intraepithelial Neoplasia 2/3 (CIN2/3)*.

NIH/OBA Receipt Date: 7-24-03. Not Selected for RAC Public Review: 8-12-03

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**0307-596 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/RNAs Transcribed In Vitro from Plasmids Containing cDNAs for MART-1, Tyrosinase, gp100, and MAGE-3/Intra-Nodal Injections**

Liao, Xinsheng M., University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Phase I Open Labeled Non-Randomized Study of RNA-DC as Tumor Vaccine in Patients with Melanoma*.

NIH/OBA Receipt Date: 7-25-03. Not Selected for RAC Public Review: 8-12-03

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**0307-597 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/Yeast Cytosine Deaminase cDNA/5-Fluorocytosine/Herpes Simplex Thymidine Kinase cDNA/Valganciclovir/Adenovirus Death Protein/Intratatumoral Injection**

Kim, Jae Ho, and Freytag, Svend, Henry Ford Health System, Detroit, Michigan; *Phase I/II Study of Replication-Competent Adenovirus-Mediated Double Suicide Gene Therapy in Combination with Salvage Intensity Modulated Radiation Therapy for the Treatment of Locally Recurrent Prostate Cancer*.

NIH/OBA Receipt Date: 7-23-03. Not Selected for RAC Public Review: 8-12-03

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**0307-598 (Open) Gene Therapy/Phase I/Infectious Disease/Adenovirus Disease/In Vitro/Adenovirus-Specific Cytotoxic T Lymphocytes/Adenovirus/No Transgene/Intravenous**

Bollard, Catherine M., and Keuhnle, Ingrid, Baylor College of Medicine, Houston, Texas; *Administration of Virus-Specific Cytotoxic T-Lymphocytes for the Prophylaxis and Therapy of Adenovirus Infection Post Allogeneic Stem Cell Transplant*.

NIH/OBA Receipt Date: 7-30-03. Not Selected for RAC Public Review: 8-19-03

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**0308-599 (Open) Gene Therapy/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta Chain cDNAs/Intravenous Infusion**

Rosenberg, Steven A., National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma by Lymphodepleting Conditioning Followed by Infusion of TCR-Gene Engineered Lymphocytes and Subsequent Peptide Immunization.*

NIH/OBA Receipt Date: 8-05-03. Not Selected for RAC Public Review: 8-25-03

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**0308-600 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection**

Kantoff, Philip, Dana-Farber Cancer Institute, Boston, Massachusetts; Hayward, Malcolm, Highlands Oncology Group, Springdale, Arkansas; Patterson, Thomas, Medical & Surgical Specialists, L.L.C., Galesburg, Illinois; Lugg, James, Wyoming Research Foundation, Inc., Cheyenne, Wyoming; Clark, Randil L., North Idaho Urology, Coeur d'Alene, Idaho; Dineen, Martin K., Atlantic Urological Associates, Daytona Beach, Florida; Karlin, Gary S., Lawrenceville Urology, Lawrenceville, New Jersey; Harper, William M., IV Urology Center of Columbus, Columbus, Georgia; McGee, James L., OSF Saint Francis Medical, Peoria, Illinois; Gittleman, Marc, South Florida Medical Research, Aventura, Florida; Bilhertz, David, Urology Associates, Nashville, Tennessee; Godschalk, Michael, VA Medical Center, Richmond, Virginia; Pittman, Walter, Urology Centers of Alabama, P.C., Homewood, Alabama; Zinner, Norman, Western Clinical Research, Inc., Torrance, California; Gange, Steven, Salt Lake Research, Salt Lake City, Utah; Kadesky, Keith, Urology Clinics of North Texas, PA and Urology Specialists & Associates, PA, Dallas, Texas; Plante, Mark K., The University of Vermont, Burlington, Vermont; Tannenbaum, Sigmund I., The Urology Center, Greensboro, North Carolina; Roper, Ronald P., Urology Associates, Marietta, Georgia; Brosman, Stanley A., Pacific Clinical Research, Marina Del Rey, California; Schiff, William, Urology Associates of Central California, Fresno, California; Hamway, Sammy, South Dayton Urological Associates, Kettering, Ohio; Hauke, Ralph, University of Nebraska Medical Center, Omaha, Nebraska; Mega, Anthony, The Miriam Hospital, Providence, Rhode Island; Leventhal, Sheryl L., Nyack Hospital, Nyack, New York; Bubley, Glenn, Beth Israel Deaconess Medical Center, Boston, Massachusetts; Glode, L. Michael, The University of Colorado Health Sciences Center, Denver, Colorado; Roland, Kendrith M., Jr., Carle Clinic Association, Urbana, Illinois; Mobley, William C., Urology Associates, P.C., Davenport, Iowa; Lilly, Michael B., Loma Linda University, Loma Linda, California; Efros, Mitchell, AccuMed Research Associates, Garden City, New York; Amin, Asim, Lombardi Cancer Research Center, Georgetown University, Washington, DC; Sharkey, Jerrold, Advanced Research Institute, Inc., New Port Richey, Florida; Mason, Terry, Prairie Medical Associates, Ltd., Chicago, Illinois; Tomera, Kevin M., Alaska Clinical Research Center, Anchorage, Alaska; Kabbinar, Fairouz, University of California, Los Angeles, Los Angeles, California; Wachs, Barton H., Atlantic Urological Medical Group, Long Beach, California; Snoy, Frederick, Urology Group of New Mexico, Albuquerque, New Mexico; Fisher, Hugh A.G., The Urological Institute of Northeastern New York, Albany, New York; Bailen, James L., Metropolitan Urology, PSC, Jeffersonville, Indiana; McMurtry, James M., Virginia Urology, Richmond, Virginia; Niku, Soheil D., Simi-San Fernando Valley Urology Associates, Granada Hills, California; Feldman, Robert A., Connecticut Clinical Research Center, Waterbury, Connecticut; Sandock, David S., Midwest Research Specialists, LLC, Milwaukee, Wisconsin; Macaluso, Joseph N., Jr., Urologic Institute of New Orleans, Gretna, Louisiana; See, William A., The Medical College of Wisconsin, Milwaukee, Wisconsin; Bidair, Mohamed, Center for Urological Research, La Mesa, California; Kaufman, Howard, Columbia University College of Physicians and Surgeons, New York, New York; DiPaola, Robert, The Cancer Institute of New Jersey, New Brunswick, New Jersey; Lewis, Nancy, Fox Chase Cancer Center, Philadelphia, Pennsylvania; Perry, David J., Washington Cancer Institute/Washington Hospital Center, Washington, DC; Waterfield, William, Franklin Square Hospital Center, Baltimore, Maryland; Chatta, Gurkamal S., University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; Nemunaitis, John; Mary Crowley Medical Research Center, Dallas, Texas; DiStefano, Alfred, Arlington Cancer Center, Arlington, Texas; *A Phase II Randomized, Double Blind, Controlled Study to Evaluate the Safety and Efficacy of PROSTVAC®-VF/TRICOM™ in Combination with GM-CSF in Patients with Androgen-Independent Adenocarcinoma of the Prostate.*

NIH/OBA Receipt Date: 8-18-03. Not Selected for RAC Public Review: 9-08-03

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**0309-601 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection**

Hall, Simon J., Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenovirus-Mediated IL-12 Gene Transduction in Patients with Radiorecurrent Prostate Cancer.*

NIH/OBA Receipt Date: 9-10-03. Not Selected for RAC Public Review: 9-30-03

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**0309-602 (Open) Gene Therapy/Cancer/Chronic Myelogenous Leukemia/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection**

Levitsky, Hyam I., Johns Hopkins University School of Medicine, Baltimore, Maryland; *K562/GM-CSF Vaccination in Combination with Imatinib Mesylate for Chronic Myeloid Leukemia.*

NIH/OBA Receipt Date: 9-23-03. Not Selected for RAC Public Review: 10-14-03

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**0309-603 (Open) Gene Therapy/Phase I/Cancer/Prostate/In Vivo/Adenovirus/Serotype 5/Tumor Necrosis Factor-Related Apoptosis-Inducing Ligand (TRAIL) cDNA/Intratumoral Injection**

Konety, Badrinath, University of Iowa, Iowa City, Iowa; *A Phase I Study of Ad5-TRAIL in Men with Clinically Organ Confined Prostate Cancer Undergoing Radical Prostatectomy.*

NIH/OBA Receipt Date: 9-25-03. Not Selected for RAC Public Review: 10-16-03

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**0309-604 (Closed) Gene Therapy/Phase IIb-III/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration**

Sanz, Mark Louis, International Heart Institute of Montana, Missoula, Montana; Bajwa, Tanvir, Heart Car Associates, LLC, Milwaukee, Wisconsin; Carter, Andrew, Providence Heart Institute, Portland, Oregon; Fischell, Tim, Borgess Medical Center, Kalamazoo, Michigan; Antolin, Rosa Ana Hernandez, Hospital Clinico Unversitario San Carlos, Madrid, Spain; Beatt, Kevin J., Hammersmith Hospital, London, United Kingdom; Coghlan, John G., Royal Free Hospital, London, United Kingdom; de Belder, Adam, Royal Sussex County Hospital, Brighton, United Kingdom; Dubiel, Jacek S., Zaklad Hermodynamiki, Krakow, Poland; Duque, Marco Antonio Pena, Instituto Nacional de Cardiologia "Ignacio Chavez"; Gil, Robert, Klinik Kardiologii Inwazyjnej, Warszawa, Poland; Hulkuri, Heikki, Oulu University Hospital, Oulu, Finland; Janessens, Stefan, University Hospital Gasthuisberg, Leuven Belgium; Mautner, Branco, Institute of Cardiology and Cardiovascular Surgery of the Favalaro Foundation, Buenos Aires, Argentina; Naslund, Ulf, Norrlans Universitetssjukhus, Umea, Sweden; Naveri, Hannu, Jorvi Hospital, Espoo, Finland; O'Brien, Timothy, University College Hospital, Galway, Ireland; Redwood, Simon R., St. Thomas' Hospital, London, United Kingdom; Rothman, Martin T., The London Chest Hospital, London, United Kingdom; Ruzyllo, Witold, Instytut Kardiologii, Warszawa, Poland; Siegel, Robert M., Advanced Cardiac Specialists, Gilbert, Arizona; Sylven, Christer, Huddinge Universitetssjukhus, Stockholm, Sweden; Thomas, Martyn R., King's College Hospital, London, United Kingdom; Uren, Neal G., Royal Infirmary of Edinburgh, Edinburgh, United Kingdom; Yia-Hertuala, Seppo, University of Kuopio, Kuopio, Finland; Archer, Stephen, University of Alberta, Edmonton, Alberta, Canada; Barbeau, Gerald, Hospital Laval, Ste-Foy Quebec, Canada; Bilodeau, Luc, Montreal Heart Institute, Montreal, Quebec, Canada; Curtis, Michael, Foothills Medical Center, Calgary, Alberta, Canada; Ducas, John, Health Sciences Centre, Winnipeg, Manitoba, Canada; Goulet, Gilles, Saint-Luc Hospital, Montreal, Quebec, Canada; Hilton, J. David, Victoria Heart Institute Foundation, Victoria, BC, Canada; Lemire, Francois, Hotel-Dieu Hospital, Montreal, Quebec, Canada; Marquis, Jean-Francis, University of Ottawa Heart Institute, Ottawa, Ontario, Canada; Niemela, D. Karl, Tampere University Hospital, Tampere, Finland; Schechtmann, Norberto, Holmes Regional Medical Center, Melbourne, Florida; Stewart, Duncan, St. Michael's Hospital, Toronto, Ontario, Canada; and Reeves, Frances, CHUM Hospital Notre Dame, Montreal, Quebec, Canada; *A Multinational Multicenter, Randomized, Double-Blind, Placebo Controlled Study to Evaluate the Efficacy and Safety of Ad5FGF-4 in Patients with Stable Angina*. Sponsor: Berlex Laboratories.

NIH/OBA Receipt Date: 9-25-03. Not Selected for RAC Public Review: 10-16-03  
Closed to enrollment: 5-14-04

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**0309-605 (Open) Gene Therapy/Cancer/Chronic Myelogenous Leukemia, Acute Myelogenous Leukemia, Myelodysplasia/Elimination of Graft-vs-Host Disease/In Vitro/Allogeneic T Lymphocytes/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous**

Kornblau, Steven, M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Study Evaluating Thymidine Kinase Transduced Lymphocytes in Conjunction with Allogeneic Transplant for the Control of Graft-vs-Host Disease and the Induction of Graft-vs-Host Malignancy for Patients with High Risk Chronic Myelogenous Leukemia, Acute Myelogenous Leukemia and Myelodysplasia*.

NIH/OBA Receipt Date: 9-26-03. Not Selected for RAC Public Review: 10-17-03

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**0310-606 (Open) Gene Therapy/Cancer/Breast or Ovarian/Immunotherapy/In Vivo/Plasmid DNA/HER2 cDNA/Intradermal Injection**

Disis, Mary, University of Washington, Seattle, Washington; *A Phase I Safety and Efficacy of a DNA Plasmid Based Vaccine Encoding the HER2/neu (HER2) Intracellular Domain in Subjects with HER2 Overexpressing Tumors*.

NIH/OBA Receipt Date: 10-2-03. Not Selected for RAC Public Review: 10-24-03

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**0310-607 (Open) Gene Therapy/Cancer/Renal Cell Carcinoma or Melanoma/Immunotherapy/In Vitro/Allogeneic Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous**

Keogh, George, and Vieweg, Johannes, Duke University Medical Center, Durham, North Carolina; *A Pilot Study of Mature, Total-Tumor-RNA-Transfected, Donor-Derived Dendritic Cell Therapy in Patients Who Have Undergone Nonmyeloablative Allogeneic Stem Cell Transplantation for Metastatic Renal Cell Carcinoma or Metastatic Melanoma*.

NIH/OBA Receipt Date: 10-2-03. Not Selected for RAC Public Review: 10-24-03

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**0310-608 (Open) Gene Therapy/Phase I/Cancer/EBV-Positive Hodgkin's or Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/LMP2A-Specific Cytotoxic T Lymphocytes (CTL)/Adenovirus/LMP2A cDNA/Intravenous Administration**

Bollard, Catherine M., Heslop, Helen, and Rooney, Cliona, Baylor College of Medicine, Houston, Texas; *Administration of LMP2A-Specific Cytotoxic T-Lymphocytes Following CD45 Antibody to Patients with Relapsed EBV-Positive Hodgkin's or Non-Hodgkin's Lymphoma*.

NIH/OBA Receipt Date: 10-6-03. Not Selected for RAC Public Review: 10-27-03

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**0310-609 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Plasmid/NY-ESO-1 cDNA/Particle-Mediated Delivery to Skin**

Altorki, Nasser, New York Presbyterian Hospital and Joan and Sanford I. Weill Medical College of Cornell University, New York, New York; *Safety and Immunological Evaluation of NY-ESO-1 Plasmid DNA (pPJV7611) Cancer Vaccine Given by Particle-Mediated Epidermal Delivery (PMED) in Patients with NY-ESO-1 or LAGE-1 Expressing Non-Small Cell Lung Cancer.*

NIH/OBA Receipt Date: 10-7-03. Not Selected for RAC Public Review: 10-28-03

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**0310-610 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Immunotherapy/In Vitro/Autologous Bone Marrow-Derived Stromal Cells (BMSC)/Plasmid DNA/Interleukin-12 cDNA/Electroporation/Stereotactic Injection or Via Intracranial Catheter**

Mikkelsen, Tom, Henry Ford Hospital, Detroit, Michigan; *A Phase I/II Study of the Treatment of Recurrent or Progressive Malignant Glioma Using Autologous Bone Marrow-Derived Stromal Cells (BMSC) Non-Virally Transduced to Express IL-12.* Sponsor: Cognate Therapeutics.

NIH/OBA Receipt Date: 10-7-03. Publicly Reviewed at the December 2003 RAC meeting

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**0310-611 (Open) Gene Therapy/Cardiovascular Diseases/Peripheral Artery Disease/In Vivo/Skeletal Myofibers/Plasmid DNA/VOP32E VEGF-A Transcription Factor cDNA/Intramuscular Injection**

Lederman, Robert, National Institutes of Health, Bethesda, Maryland; *Modulation of Vascular Endothelial Growth Factor (VEGF) Using an Engineered Zinc-Finger Transcription Factor to Treat Lower Limb Intermittent Claudication.*

NIH/OBA Receipt Date: 10-7-03. Not Selected for RAC Public Review: 10-28-03

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**0310-612 (Open) Gene Therapy/Phase II/Immunotherapy/In Vitro/Cancer/Non-Small Cell Lung Carcinoma (NSCLC)/Bronchioloalveolar Carcinoma (BAC)/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intradermal Injections**

Davies, Angela M., University of California, Davis, Sacramento, California; and Mudad, Raja; Tulane University; New Orleans, Louisiana; *Phase II Trial of CG8123, an Autologous Cancer Vaccine (GVAX), in Patients with Selected Stage IIIB and IV Bronchioloalveolar Carcinoma (BAC).* Sponsor: Southwest Oncology Group.

NIH/OBA Receipt Date: 10-24-03. Not Selected for RAC Public Review: 11-14-03

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**0311-613 (Under review) Gene Therapy/Phase I/Cancer/Sarcoma or Neuroblastoma/In Vivo/Herpes Simplex Virus Type 1/Vector-Directed Tumor Cell Lysis/Rat Prodrug Enzyme CYP2B1 cDNA/Intratumoral Injection**

Cripe, Timothy P., Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio; *A Phase I Dose Escalation Study of Intratumoral Herpes Simplex Virus-1 Mutant in Patients with Refractory Sarcoma or Neuroblastoma.*

NIH/OBA Receipt Date: 11-6-03. Review at a RAC meeting pending; investigators have requested postponement of public review.

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**0311-614 (Open) Non-therapeutic (Healthy Volunteers)/Dental Caries Prevention/In Vivo/Streptococcus mutans/Oral Administration**

Stone, Constance E., University of Florida, Gainesville, Florida; *First Time in Human Safety Study of Streptococcus mutans Lactic Acid-Deficient Effector Strain (A2JM) Administered in Conjunction with Twice Daily Dose of D-alanine Mouthwash in Healthy Adult Male Subjects for Replacement Therapy as an Aid in the Protection against Dental Caries.* Sponsor: Oragenics, Inc.

NIH/OBA Receipt Date: 11-20-03. Publicly Reviewed at the March 2004 RAC meeting

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**0311-615 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/gp100 Melanoma Antigen/Intramuscular Administration of a Nonmyeloablative Lymphocyte Depleting Regimen**

Rosenberg, Steven A., National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma using Lymphocytes Reactive with the GP100 Antigen following the Administration of a Nonmyeloablative Lymphocyte Depleting Regimen.*

NIH/OBA Receipt Date: 11-25-03. Not Selected for RAC Public Review: 12-16-03

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**0312-616 (Open) Gene Therapy/Phase I-II/Cancer/Colon Carcinoma (Hepatic Metastasis)/Herpes Simplex Virus Type-1/Vector-Directed Cell Lysis/Intrahepatic Artery Administration**

Fong, Yuman; Memorial Sloan-Kettering Cancer Center, New York, New York; and Nemunaitis, John J.; Mary Crowley Medical Research Center, Dallas, Texas; *A Phase I/II, Open-Label Study (with a Sequential Dose Escalation Stage followed by an Expansion of a Selected Dose Cohort), to Evaluate the Safety and Anti-Tumor Effects of NV1020, Administered Repeatedly Via Hepatic Artery Infusion Prior to Second-Line Chemotherapy, in Patients with Colorectal Adenocarcinoma Metastatic to the Liver.* Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 1-13-04. Not Selected for RAC Public Review: 2-3-04

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**0312-617 (Open) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vivo/Plasmid DNA/Human and Mouse Prostate Specific Membrane Antigen cDNAs/Intramuscular Injection**

Slovin, Susan F., Memorial Sloan-Kettering Cancer Center, New York, New York; *Injection of Renal Cell Carcinoma Patients with Human and Mouse Prostate Specific Membrane Antigen (PSMA) DNA: A Phase I Trial to Assess Safety and Immune Response.*

NIH/OBA Receipt Date: 12-9-03. Not Selected for RAC Public Review: 12-30-03

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**0312-618 (Open) Gene Therapy/Phase I/Cancer/CEA Expressing Cancers/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection**

Marshall, John, Georgetown University Medical Center, Washington, D.C.; *Randomized Single Institution Pilot Study of Vaccinia-CEA(6D0-TRICOM with GM-CSF in Combination with Docetaxel in Patients with CEA-Bearing Cancers.*

NIH/OBA Receipt Date: 12-18-03. Not Selected for RAC Public Review: 2-11-04

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**0312-619 (Open) Gene Therapy/Monogenic Disease/ Late Infantile Neuronal Ceroid Lipofuscinosis/In Vivo/Brain/Adeno-Associated Virus 2/CLN2 (Tripeptidyl Peptidase) cDNA/Intraparenchymal Injection**

Crystal, Ronald, Weill Medical College of Cornell University, New York, New York; *Administration of a Replication Deficient Adeno-Associated Virus Gene Transfer Vector Expressing the Human CLN2 cDNA to the Brain of Children with Late Infantile Neuronal Ceroid Lipofuscinosis.*

NIH/OBA Receipt Date: 12-29-03. Not Selected for RAC Public Review: 1-20-04

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**0312-620 (Open) Gene Therapy/Phase I/Cancer/Squamous Cell Carcinoma of the Head and Neck/Radiotherapy/In Vivo/Adenovirus/Serotype 5/Human Sodium-Iodide Symporter (NIS) cDNA/Intratumoral Injection**

Trask, Douglas K. and Domann, Frederick E., Carver College of Medicine, The University of Iowa, Iowa City, Iowa; *A Phase I Study of Genetically Targeted Radiotherapy using Intratumoral Administration of Adenovirus Expressing the Sodium-Iodine Symporter (Ad-NIS) Plus Systemic <sup>131</sup>I in Subjects with Refractory Squamous Cell Carcinoma of the Head and Neck.*

NIH/OBA Receipt Date: 12-30-03. Not Selected for RAC Public Review: 1-21-04

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**0401-621 (Open) Gene Therapy/Phase I/Cancer/Advanced Myelodysplasia (MDS) or Refractory Acute Myeloid Leukemia (AML)/Immunotherapy/In Vitro/Autologous Myeloblasts/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous Injection**

Ho, Vincent T., Dana-Farber Cancer Institute and Harvard Medical School, Boston, Massachusetts; *GM-CSF Secreting Leukemia Cell Vaccinations after Allogeneic Non-Myeloablative Peripheral Blood Stem Cell Transplantation in Patients with Advanced Myelodysplastic Syndrome or Refractory Acute Myeloid Leukemia.*

NIH/OBA Receipt Date: 1-6-04. Not Selected for RAC Public Review: 1-27-04

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**0401-622 (Open) Gene Therapy/Phase I-II/Other Diseases-Disorders/Congestive Heart Failure (CHF)/In Vivo/Adenovirus/Serotype 5/Adenylyl Cyclase Type VI (AC<sub>VI</sub>) cDNA/Intracoronary Injection**

Hammond, H. Kirk, University of California, San Diego, San Diego, California; *Adenylyl Cyclase VI Gene Transfer for CHF.*

NIH/OBA Receipt Date: 1-9-04. Publicly Reviewed at the March 2004 RAC meeting

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**0401-623 (Open) Gene Therapy/Phase I-II/Other Diseases-Disorders/Alzheimer's Disease/In Vivo/Adeno-Associated Virus/Serotype 2/Nerve Growth Factor cDNA/Intracranial Injection**

Bennett, David A., Rush University Medical Center, Chicago, Illinois; *A Phase I/II, Dose-Escalating, Randomized and Controlled Study to Assess the Safety, Tolerability, and Efficacy of CERE-110 [Adeno-Associated Virus (AAV)-based, Vector-Mediated Delivery of beta-Nerve Growth Factor (NGF)] in Subjects with Mild to Moderate Alzheimer's Disease.* Sponsor: Ceregene, Inc.

NIH/OBA Receipt Date: 1-12-04. Publicly Reviewed at the March 2004 RAC meeting

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**0401-624 (Open) Gene Therapy/Phase I-II/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Adenovirus/Serotype 5/Replication-Competent Virus/RGD Motif/Intratumoral Injection**

Lang, Frederick F. and Conrad, Charles A., The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Phase I Trial of Conditionally Replication-Competent Adenovirus (Delta-24-RGD) for Recurrent Malignant Gliomas.*

NIH/OBA Receipt Date: 1-12-04. Publicly Reviewed at the March 2004 RAC meeting

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**0401-625 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Vector-Directed Cell Lysis/In Vivo/Adenovirus/Serotype 5/Replication –Competent Virus/RGD Motif/Intraperitoneal Injection**

Alvarez, Ronald D., Barnes, Mack N., and Curiel, David T., University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Study of a Tropism Modified Conditionally Replicative Vector (Ad5-D24RGD) for Intraperitoneal Delivery in Ovarian and Extraovarian Cancer Patients.*

NIH/OBA Receipt Date: 1-13-04. Publicly Reviewed at the March 2004 RAC meeting

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**0401-626 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vivo/Plasmid in Poly (DL-lactide-coglycolide) (PLG) Microparticles/Cytochrome P450 Isoenzyme 1B1 (CYP1B1) cDNA/Subcutaneous Injection**

Gribben, John G., Dana-Farber Cancer Institute and Harvard Medical School, Boston, Massachusetts; *A Phase I Study to Determine the Feasibility and Safety of Vaccinating Cancer Patients with ZYC300 in Combination with GM-CSF and Imiquimod with or without Cyclophosphamide Pre-Dosing.*

NIH/OBA Receipt Date: 1-13-04. Not Selected for RAC Public Review: 2-3-04

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**0401-627 (Open) Gene Therapy/Phase I-II/Cancer/Epstein-Barr Virus Lymphoma/Pro-Drug/Elimination of Graft Versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous**

O'Reilly, Richard J., Memorial Sloan-Kettering Cancer Center, New York, New York; *A Phase I-II Trial of EBV-Sensitized Donor T-Cells Transduced with a Dicistronic Retroviral Vector, Termed NIT, Encoding a Mutant Human Nerve Growth Factor Receptor (mLNGFR) and Herpes Simplex Virus Thymidine Kinase (HSV-TK) for the Treatment of EBV Lymphoma in Marrow and Organ Allograft Recipients, and in Patients with Genetic Immunodeficiencies.*

NIH/OBA Receipt Date: 1-13-04. Not Selected for RAC Public Review: 2-3-04

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**0401-628 (Open) Gene Therapy/Other Diseases-Disorders/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Percutaneous Cardiac Catheterization/Intra-Myocardial Injection**

Losordo, Douglas W., St. Elizabeth's Medical Center, Boston, Massachusetts; *An Evaluation of a 800 µg dose of pVG1.1 (VEGF2) Percutaneously Delivered Via Boston Scientific Corporation Stiletto™ Endocardial Injection Catheter in up to Seven Angina Patients Who Previously Received a Placebo Dose within Study Protocol VEGF2-CAD-CL-005.* Sponsor: Corautes Genetics, Inc.

NIH/OBA Receipt Date: 1-13-04. Not Selected for RAC Public Review: 2-3-04

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**0401-629 (Open) Gene Therapy/Phase I/Cancer/Superficial Injectable Tumors/Vaccinia Virus/Vector-Directed Tumor Lysis/Pro-Drug/Cytosine Deaminase cDNA/5-FC/Somatostatin Receptor cDNA/Intratumoral Injection**

Bartlett, David L., University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *A Phase I Dose-Escalation Trial of vvDD-CDSR (Double-Deleted Vaccinia Virus Plus CD/SMR) Administered by Intratumoral Injection in Patients with Superficial Injectable Tumors.*

NIH/OBA Receipt Date: 1-13-04. Publicly Reviewed at the March 2004 RAC meeting

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**0401-630 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Human Telomerase Reverse Transcriptase (hTERT) cDNA/Intradermal Injections**

Dahm, Philipp and Vieweg, Johannes, Duke University Medical Center, Durham, North Carolina; *Active Immunotherapy with Mature, Human Telomerase Reverse Transcriptase RNA-Transfected, Autologous Dendritic Cells with or without the IL-2 Diphtheria Toxin Conjugate Denileukin Diftox (ONTAK<sup>R</sup>) in Subjects with Metastatic Prostate Cancer.*

NIH/OBA Receipt Date: 1-16-04. Not Selected for RAC Public Review: 2-6-04

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**0401-631 (Open) Gene Therapy/Phase I/Cancer/CEA Expressing Cancers/Liver Metastasis/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection**

Gulley, James, National Institutes of Health, Bethesda, Maryland and Hoffmeister, Karen, National Naval Medical Center, Bethesda, Maryland; *A Phase I Trial of a CEA-TRICOM Based Vaccine and Radiation to Liver Metastasis with or without Celecoxib in Patients with CEA Positive Solid Tumors.*

NIH/OBA Receipt Date: 1-21-04. Not Selected for RAC Public Review: 2-10-04

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**0403-632 (Open) Gene Therapy/Phase I-II/Diabetic Peripheral Neuropathy/In Vivo/Plasmid/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection**

Losordo, Douglas W., St. Elizabeth's Medical Center, Boston, Massachusetts; *pVGI.1 (VEGF-2) Gene Transfer for Diabetic Neuropathy.*

NIH/OBA Receipt Date: 3-18-04. Not Selected for RAC Public Review: 4-7-04

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**0403-633 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Multiple Sclerosis (MS)/Immunotherapy/In Vivo/Plasmid DNA/Myelin Basic Protein (hMBP) cDNA/Intramuscular Injection**

Vollmer, Timothy L., Barrow Neurological Institute, St. Joseph's Hospital and Medical Center, Phoenix, Arizona; *Phase I Trial of Immunotherapy with BHT-3009 Alone or Combined with Atorvastatin in Patients with Multiple Sclerosis.* Sponsor: Bayhill Therapeutics

NIH/OBA Receipt Date: 3-22-04. **Publicly Reviewed at the June2004 RAC meeting**

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**0403-634 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/B7.1 (CD80)/ICAM-1/LFA-3/Intratumoral Injection**

Gajewski, Thomas F., University of Chicago Medical Center, Chicago, Illinois; *Phase II Study of Intratumoral Injection of rF-TRICOM<sup>TM</sup> in Patients with Metastatic Melanoma Who have Detectable Circulating Melanoma-Specific T Cells.*

NIH/OBA Receipt Date: 3-9-04. Not Selected for RAC Public Review: 3-29-04

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**0403-635 (Open) Gene Therapy/Phase III/Cancer/Pancreas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection**

Marshall, John, Lombardi Cancer Research Center, Georgetown University Medical Center, Washington, DC; DiStefano, Alfred, Arlington Cancer Center, Arlington, Texas; Morse, Michael; Duke University Medical Center, Durham, North Carolina; Gurtler, Jayne; Gurtler and Brinz, APMC, Metairie, Louisiana; Nemunaitis, John; Mary Crowley Medical Research Center, Dallas, Texas; Rowland, Kendrith; Carle Cancer Center, Urbana, Illinois; Geils, George F. Jr.; Charleston Hematology-Oncology, PA, Charleston, South Carolina; Hagan, M. Kelly; Virginia Cancer Institute, Richmond, Virginia; Langdon, Robert M.; Nebraska Methodist Hospital, Omaha, Nebraska; Lenz, Heinz-Josef; USC/Norris Comprehensive Cancer, Los Angeles, California; Eisenberg, Peter; California Cancer Care, Greenbrae, California; Holladay, Charles S.; Charleston Cancer Center, Charleston, South Carolina; and Ervin, Thomas J., Maine Center for Cancer Medicine and Blood Disorders, Scarborough, Maine; *A Phase III Randomized, Controlled Study to Evaluate the Safety and Efficacy of PANVAC<sup>TM</sup>-VF in Combination with GM-CSF Versus Best Supportive Care of Palliative Chemotherapy in Patients with Metastatic (Stage IV) Adenocarcinoma of the Pancreas Who Have Failed a Gemcitabine-Containing Chemotherapy Regimen.* Sponsor: Therion Biologics Corporation

NIH/OBA Receipt Date: 3-8-04. Not Selected for RAC Public Review: 3-29-04

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**0403-636 (Open) Gene Therapy/Phase I/Cancer/Colorectal Adenocarcinoma/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection**

Gulley, James L., National Institutes of Health, Bethesda, Maryland; *An Open Label Pilot Study to Evaluate the Safety and Tolerability of PANVAC<sup>TM</sup>-V and PANVAC<sup>TM</sup>-F in Combination with Sargramostim in Patients with Metastatic Colorectal Adenocarcinoma.*

NIH/OBA Receipt Date: 3-26-04. Not Selected for RAC Public Review: 4-15-04

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**0403-637 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Adenovirus Type 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intravenous Injection**

Small, Eric, UCSF Comprehensive Cancer Center, San Francisco, California; and Senzer, Neil; Mary Crowley Medical Research Center, Dallas, Texas; *A Phase 1/2a Dose-Escalation Trial of Intravenous CG7870 in Combination with Docetaxel in Chemotherapy-Naïve Patients with Metastatic Hormone-Refractory Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 3-29-04. Not Selected for RAC Public Review: 4-16-04

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**0404-638 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Alpha-1 Antitrypsin Deficiency/In Vivo/Adeno-Associated Virus/Serotype 1/Alpha-1 Antitrypsin cDNA/Intramuscular**

Flotte, Terence R., University of Florida, Gainesville, Florida; *Phase I Trial of Intramuscular Injection of a Recombinant Adeno-Associated Virus 1-Alpha 1-Antitrypsin (rAAV-CB-hAAT) Gene Vector to AAT-Deficient Adults*.

NIH/OBA Receipt Date: 4-6-04. **Publicly Reviewed at the June2004 RAC meeting**

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**0404-639 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/ $\alpha(1,3)$  galactosyltransferase Gene/Subcutaneous Injection**

Hemstreet, George P., University of Nebraska Medical Center, Omaha, Nebraska; *A Phase I/II Study of an Antitumor Vaccination using  $\alpha(1,3)$  galactosyltransferase Expressing Allogeneic Tumor Cells in Patients with Hormone Refractory Prostate Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

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**0404-640 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/ $\alpha(1,3)$  galactosyltransferase Gene/Intradermal Injection**

Morris, John C., National Institutes of Health, Bethesda, Maryland; *A Phase I/II Study of an Antitumor Vaccination using  $\alpha(1,3)$  galactosyltransferase [ $\alpha(1,3)$  GT] Expressing Allogeneic Tumor Cells in Patients with Recurrent or Refractory Malignant Melanoma*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

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**0404-641 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Colorectal, Pancreatic, and Non-Small Cell Lung Cancers/Immunotherapy/In Vivo/*Saccharomyces cerevisiae*/Mutated Ras Oncoproteins/Subcutaneous Injections**

O'Neil, Bert, University of North Carolina School of Medicine, Chapel Hill, North Carolina; *A Phase I Open Label, Non-Randomized, Dose-Escalation, Multi-Center, Therapeutic Trial of the Safety, Immunogenicity and Efficacy of GI-4000, an Inactivated Recombinant *Saccharomyces cerevisiae* Immunotherapeutic Expressing Three Different Mutations of the Ras Oncoprotein, in Patients with Solid Tumors Expressing Mutations in Ras*. Sponsor: GlobelImmune, Inc.

NIH/OBA Receipt Date: 4-13-04. **Publicly Reviewed at the June2004 RAC meeting**

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**0404-642 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Heart Failure/In Vivo/Adeno-Associated Virus/Serotype 6/Sarcoplasmic Reticulum Calcium ATPase 2a (SERCA2a) cDNA/Intramyocardial Injection**

London, Barry, University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *Phase I Trial of Gene Transfer During VD Support with SERCA2a*.

NIH/OBA Receipt Date: 4-13-04. **Publicly Reviewed at the June2004 RAC meeting**

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**0404-643 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Bladder/Vector-Directed Cell Lysis/In Vivo/Adenovirus Serotype 5/Conditionally Replication Competent Virus/GM-CSF/Intravesical Administration**

Nemunaitis, John, US Oncology, Dallas, Texas; *A Phase I/II Dose-Escalation Trial of Intravesical CG0070 for Superficial Transitional Cell Carcinoma of the Bladder after Bacillus Calmette-Guerin Failure*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 4-13-04. **Publicly Reviewed at the June2004 RAC meeting**

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**0404-644 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Adenovirus Serotype 5/Fusion Protein of MUC-1 and CD40 Ligand (CD40L) cDNA/Subcutaneous Injection**

Deisseroth, Albert B., Sidney Kimmel Cancer Center, San Diego, California; *A Single Arm Open-Label Phase I Study of an Injectable Replication-Incompetent Adenoviral Vector Vaccine with Protein Boost Used to Produce an Immune Response for MUC-1 Positive Epithelial Cancer Cells in Advanced Breast Cancer Patients.*

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

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**0404-645 (Open) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/Plasmid/Fibroblast Growth Factor-1 (FGF-1) cDNA/Intramuscular Injections**

Henry, Tim, Minneapolis Heart Institute Foundation, Minneapolis, Minnesota; *Double-Blind, Randomized, Placebo-Controlled, Parallel Group and Dose-Finding, Multicentric, Safety and Efficacy Study with Intramuscular Injections of NV1FGF in Subjects with Intermittent Claudication.* Sponsor: Gencell

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

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**0404-646 (Open) Gene Therapy/Phase I-II/Infectious Diseases/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Lentivirus/HIV-1/Antisense env/Intravenous**

Stein, David, Albert Einstein College of Medicine, Bronx, New York; *A Phase I/II, Open-Label, Multicenter Study to Evaluate the Safety, Tolerability, and Therapeutic Effect of Repeated Doses of Autologous T Cells Transduced with VRX496 in Antiretroviral Experienced HIV-Positive Patients.* Sponsor: VIRxSYS

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-10-04

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**0404-647 (Open) Gene Therapy/Phase II/Cancer/Superficial Injectable Tumors/Vaccinia Virus/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) and Humanized Escherichia coli  $\beta$  galactosidase cDNAs/Intratumoral Injection**

Nemunaitis, John, US Oncology, Dallas, Texas; *A Phase I/II Dose-Escalation Trial of JX-594 (Thymidine Kinase-Deleted Vaccinia Virus Plus GM-CSF) Administered by Intratumoral Injection in Patients with Superficial Injectable Tumors.* Sponsor: Jennerex Biotherapeutics, Inc.

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

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**0404-648 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous and Intraprostatic Injections**

Gulley, James L., National Institutes of Health, Bethesda, Maryland; *A Phase I Feasibility Study of an Intraprostatic PSA-Based Vaccine in Prostate Cancer Patients with Local Failure Following Radiotherapy.*

NIH/OBA Receipt Date: 4-19-04. Not Selected for RAC Public Review: 5-7-04

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**0404-649 (Open) Gene Therapy/Phase II/Cancer/Hepatic Metastasis of Colorectal Adenocarcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injections**

Morse, Michael, Duke University Medical Center, Durham, North Carolina; *A Phase II Study of Active Immunotherapy with PANCACTM or Autologous Cultured Dendritic Cells Infected with PANVAC<sup>TM</sup> After Complete Resection of Hepatic Metastasis of Colorectal Carcinoma.*

NIH/OBA Receipt Date: 4-27-04. Not Selected for RAC Public Review: 5-17-04

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**0405-650 (Open) Gene Therapy/Phase II/Cancer/Immunotherapy/In Vivo/Vaccinia/Fowlpox virus/NY-ESO-1 cDNA/Subcutaneous Injection**

Odunsi, Kunle O., Roswell Park Cancer Institute and State University of New York at Buffalo, Buffalo, New York; *Phase II Study of Recombinant Vaccinia-NY-ESO-1 (rV-NY-ESO-1) and Recombinant Fowlpox-Ny-ESO-1 (rF-NY-ESO-1) in Patients with Epithelial Ovarian, Fallopian Tube or Primary Peritoneal Carcinoma Whose Tumors Express NY-ESO-1 or LAGE-1 Antigen.*

NIH/OBA Receipt Date: 5-6-04. Not Selected for RAC Public Review: 6-4-04

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**0405-651 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD 80)/ICAM-1/LFA-3/Subcutaneous Injection**

Arlen, Philip M., National Institutes of Health, Bethesda, Maryland; *A Pilot Trial of a CEA/TRICOM-Based Vaccine in Combination with Combined Chemotherapy/Radiotherapy in Patients with Unresectable Stage III Non-Small Cell Lung Cancer (NSCLC).*

NIH/OBA Receipt Date: 5-13-04. Not Selected for RAC Public Review: 6-4-04

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**0405-652 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vivo/Plasmid DNA/HPV16 E7 cDNA/Intramuscular Injection**

Gillison, Maura L., Johns Hopkins Comprehensive Cancer Center, Baltimore, Maryland; *An Open-Label Phase One Study of the Safety and Immunogenicity of Repeated Vaccination with NGVL4a-HPV16Sig/E7(detox)/HSP70 in Patients with Stage III or IV HPV 16-Positive Head and Neck Squamous Cell Carcinoma (HNSCC).*

NIH/OBA Receipt Date: 5-18-04. Not Selected for RAC Public Review: 6-8-04

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**0405-653 (Open) Gene Therapy/Phase III/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection**

Urba, Walter J., Providence Portland Medical Center, Portland, Oregon; *A Phase III Randomized, Open-Label Study of CG1940 and CG8711 Versus Docetaxel and Estramustine in Patients with Metastatic Hormone-Refractory Prostate Cancer Who are Chemotherapy-Naïve.* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 5-20-04. Not Selected for RAC Public Review: 6-10-04

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**0406-654 (Open) Gene Therapy/Cancer/Mantle Cell Lymphoma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/Electroporation/DMRIE-Cholesterol/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/CD40 Ligand cDNA/Intradermal Injection**

Dessureault, Sophie, The Moffitt Cancer Center and Research Institute, University of South Florida, Tampa, Florida; *A Phase II Trial Using a Universal GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) in the Formulation of Autologous Tumor Cell-Based Vaccines for Patients with Mantle Cell Lymphoma.*

NIH/OBA Receipt Date: 6-1-04. Not Selected for RAC Public Review: 6-22-04

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**0406-655 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Human Telomerase Reverse Transcriptase (hTERT)/Intradermal Injections**

Dahm, Philipp, Duke University Medical Center, Durham, North Carolina; *Active Immunotherapy with Human LAMP Telomerase mRNA-Transfected Immature, Autologous Dendritic Cells Following In-Situ Priming with the Immunostimulant Imiquimod (ALDARA™) in Patients with Metastatic Prostate Cancer.*

NIH/OBA Receipt Date: 6-9-04. Not Selected for RAC Public Review: 6-29-04

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**0407-656 (Open) Gene Therapy/Phase I-II/Other/Diabetic Peripheral Neuropathy/In Vivo/Skeletal Muscle Myocytes/Plasmid DNA/VOP32E VEGF-A Transcription Factor cDNA/Intramuscular Injection**

Cornblath, David, Johns Hopkins Hospital, Baltimore, Maryland; *A Phase I/II, Dose-Escalation Clinical Trial of SB509 in Subjects with Diabetic Neuropathy.* Sponsor: Sangamo BioSciences, Inc.

NIH/OBA Receipt Date: 7-6-04. Not Selected for RAC Public Review: 7-27-04

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**0407-657 (Open) Gene Therapy/Phase II/Cancer/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/Electroporation/DMRIE-Cholesterol/Granulocyte-Macrophage Colony Stimulating Factor cDNA/CD40 Ligand cDNA/Intradermal Injection**

Dessureault, Sophie, H. Lee Moffitt Cancer Center & Research Institute, University of South Florida, Tampa, Florida; *A Phase II Trial Using a Universal GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) in the Formulation of Autologous Tumor Cell-Based Vaccines for Patients with Malignant Melanoma.*

NIH/OBA Receipt Date: 7-12-04. Not Selected for RAC Public Review: 8-2-04

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**0407-658 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid DNA/Prostatic Acid Phosphatase (PAP) cDNA/Intradermal Injection**

McNeel, Douglas G.; University of Wisconsin Medical School, Madison, Wisconsin; *A Phase I Study of a DNA-Based Vaccine Targeting Acid Phosphatase (PAP) in Patients with Stage D0 Prostate Cancer.*

NIH/OBA Receipt Date: 7-23-04. Not Selected for RAC Public Review: 8-12-04

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**0407-659 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vitro/Allogeneic Fibroblasts/DNA-Liposome Complexes/Tumor Cell DNA Fragments/Intradermal Injection**

Cohen, Edward P.; University of Illinois at Chicago; Chicago, Illinois; *Active Immunization of Patients with Stage III/IV Carcinoma of Oral Cavity or Oropharynx with Semi-Allogeneic Non Malignant Human Fibroblasts Transfected with DNA from Autologous Tumor (Phase I Study).*

NIH/OBA Receipt Date: 7-26-04. Not Selected for RAC Public Review: 8-16-04

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**0407-660 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Fusion Protein of MUC-1 and CD40 Ligand (CD40L) cDNA/Subcutaneous Injection**

Deisseroth, Albert; Sidney Kimmel Cancer Center; San Diego, California; *A Single Arm Open-Label Phase I Study of an Injectable Replication-Incompetent Adenoviral Vector Vaccine with Protein Boost used to Produce an Immune Response for MUC-1 Positive Epithelial Cancer Cells in Prostate Cancer Patients.*

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-661 (Open) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1/VP16 cDNA/Intramuscular Injection**

Olin, Jeffrey W.; Mount Sinai School of Medicine; New York, New York; *A Phase 2, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, Multicenter, Dose-Selection Study of Ad2/Hypoxia Inducible Factor (HIF)-1  $\alpha$ /VP16 in Patients with Intermittent Claudication.* Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-662 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag cDNA/Interleukine-15 cDNA/Intramuscular Injections**

Baden, Lindsey Robert and Dolin, Raphael; Harvard Medical School and Brigham and Women's Hospital; Boston, Massachusetts; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of HIV-1 Gag DNA alone or with IL-15 DNA, Boosted with Homologous Vaccine, HIV CTL Multi-Epitope (MEP) Vaccine, or Gag + IL-12 Plasmid Vaccine in Healthy, HIV-1 Uninfected Adult Participants.*

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-663 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag cDNA/Interleukine-12 cDNA/Intramuscular Injections**

Wright, Peter Farnum and Spearman, Paul W.; Vanderbilt University School of Medicine; Nashville, Tennessee; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of an HIV-1 Gag DNA Vaccine with or without IL-12 DNA Adjuvant, Boosted with Homologous Plasmids or with HIV CTL Multiepitope Vaccine / RC529-SE Plus GM-CSF, in Healthy, HIV-1 Uninfected Adult Participants.*

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-664 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag cDNA/Interleukine-12 cDNA/Intramuscular Injections**

Wright, Peter Farnum and Spearman, Paul W.; Vanderbilt University School of Medicine; Nashville, Tennessee; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of HIV-1 Gag DNA Vaccine and IL-12 DNA Adjuvant or HIV CTL Multiepitope Peptide Vaccine / RC529-SE with or without GM-CSF Given to Participants Who have Received the CTL MEP / RC529-SE Vaccine with or without GM-CSF as a Priming Regimen.*

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-665 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Herpes Simplex Virus Type-1/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intratumoral Injection**

Nemunaitis, John; Mary Crowley Medical Research Center; Dallas, Texas; *A Phase II Study of the Efficacy, Safety and Immunogenicity of ONCOVEX<sup>GM-CSF</sup> in Patients with Inoperable Malignant Melanoma*. Sponsor: BioVex, Limited

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-666 (Open) Gene Therapy/Phase I-II/Cancer/Colorectal Carcinoma with Liver Metastasis/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interferon-beta/Intravenous Administration**

Reid, Tony; University of California, San Diego; San Diego, California; *A Phase I/II Study of Interferon-beta Gene Transfer (Ad.hIFN- $\beta$ ) in the Treatment of Refractory Colorectal Carcinoma with Liver Metastasis*. Sponsor: Biogen Idec Inc.

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-667 (Open) Gene Therapy/Phase I-II/Infectious Diseases/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Lentivirus/HIV-1/Antisense env/Intravenous**

June, Carl and MacGregor, Rob Roy; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Phase I/II, Open-Label, Single Center Study to Evaluate the Tolerability, Trafficking and Therapeutic Effects of Repeated Doses of Autologous T Cells Transduced with VRX496 in HIV-Infected Subjects*. Sponsor: VIRxSYS Corporation

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-668 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/p501 cDNA/Intratumoral Injection**

Higano, Celestia S.; University of Washington, Seattle Cancer Care Alliance; Seattle, Washington; *Immunotherapy Consisting of Adenovirus Expressing P501 Protein Followed by CPC-P501 Protein Plus AS15 Adjuvant: A Phase I/II Trial of a Therapeutic Vaccine Regimen in Patients with Prostate Cancer who have PSA  $\geq$  0.4 ng/ml after Radical Prostatectomy*. Sponsor: Corixa Corporation

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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**0407-669 (Under review) Gene Therapy/Phase I/Other Diseases-Disorders/Epilepsy/In Vivo/Adeno-Associated Virus/Pseudotype 1 /2/Human Neuropeptide Y (NPY) cDNA/Intracranial Injection**

During, Matthew, J.; Weill Medical College of Cornell University, New York, New York; and Fried, Itzhak and Stern, John M.; University of Los Angeles, California School of Medicine; Los Angeles, California; *Hippocampal NPY Gene Transfer in Subjects with Intractable Temporal Lobe Epilepsy*.

NIH/OBA Receipt Date: 7-30-04.

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**(Scroll down for Summary Table)**



<b>HUMAN GENE TRANSFER PROTOCOLS</b> (please see explanation of review levels below)								
	Review Level 1	Review Level 2	Review Level 3	Review Level 4	Review Level 5	Review Level 6	Review Level 7	TOTAL
<b>MARKING</b>	23	2	5	0	0	10	1	41
<b>THERAPY</b>	83	5	92	5	8	334	80	607
<b>NON-THERAPEUTIC</b>	1	0	0	0	0	4	4	9
<b>INFECTIOUS DISEASES</b>	8	1	11	1	1	18	2	42
1. Human Immunodeficiency Virus	8	1	11	1	1	15	2	39
2. Other viral diseases	0	0	0	0	0	3	0	3
<b>MONOGENIC DISEASES</b>	20	1	9	0	1	16	12	59
1. Alpha-1-Antitrypsin Deficiency	1	0	0	0	0	1	1	3
2. Chronic Granulomatous Disease	1	0	1	0	0	1	0	3
3. Cystic Fibrosis	10	1	5	0	0	6	1	23
4. Familial Hypercholesterolemia	1	0	0	0	0	0	0	1
5. Fanconi Anemia	1	0	0	0	0	3	0	4
6. Gaucher Disease	3	0	0	0	0	0	0	3
7. Hunter Syndrome	1	0	0	0	0	0	0	1
8. Ornithine Transcarbamylase Deficiency	0	0	1	0	0	0	0	1
9. Purine Nucleoside Phosphorylase Deficiency	1	0	0	0	0	0	0	1
10. SCID	1	0	1	0	0	3	1	6
11. Leukocyte Adherence Deficiency	0	0	1	0	0	0	0	1
12. Canavan Disease	0	0	0	0	0	1	2	3
13. Hemophilia	0	0	0	0	0	0	5	5
14. Muscular Dystrophy	0	0	0	0	0	0	1	1
15. Amyotrophic Lateral Sclerosis	0	0	0	0	1	0	0	1
16. Junctional Epidermolysis Bullosa	0	0	0	0	0	0	1	1
17. Neuronal Ceroid Lipofuscinosis	0	0	0	0	0	1	0	1
<b>OTHER DISEASES / DISORDERS</b>	2	0	2	2	3	38	29	76
1. Peripheral Artery Disease	1	0	0	1	1	20	4	27
2. Arthritis	1	0	0	0	0	0	3	4
3. Arterial Restenosis	0	0	1	0	0	1	1	3
4. Heart Failure	0	0	0	0	0	0	2	2
5. Cubital Tunnel Syndrome	0	0	1	0	0	0	0	1
6. Coronary Artery Disease	0	0	0	1	0	15	5	21
7. Alzheimer's Disease	0	0	0	0	0	0	2	2
8. Ulcer	0	0	0	0	0	1	2	3
9. Bone Fracture	0	0	0	0	1	0	0	1
10. Peripheral Neuropathy	0	0	0	0	0	1	1	2
11. Parkinson's Disease	0	0	0	0	0	0	2	2
12. Epilepsy	0	0	0	0	1	0	0	1
13. Eye Disorders	0	0	0	0	0	0	4	4
14. Erectile Dysfunction	0	0	0	0	0	0	1	1
15. Intractable Pain	0	0	0	0	0	0	1	1
16. Autoimmune Disease	0	0	0	0	0	0	1	1
<b>CANCER (BY THERAPEUTIC APPROACH)</b>	53	3	70	2	3	262	37	430
1. Antisense	4	0	0	0	0	3	0	7
2. Chemoprotection	4	0	4	0	0	4	0	12
3. Immunotherapy/In Vitro Transduction	22	2	19	0	0	76	6	125
4. Immunotherapy/In Vivo Transduction	7	0	28	1	2	112	8	158
5. Pro-drug/HSV-TK and Ganciclovir	12	1	10	0	1	14	6	44
6. Tumor Suppressor Gene	3	0	6	0	0	26	3	38
7. Single Chain Antibody	0	0	2	0	0	0	0	2
8. Oncogene Down-Regulation	1	0	1	1	0	7	0	10
9. Vector-Directed Cell Lysis	0	0	0	0	0	18	10	28
10. Dominant Negative Mutation	0	0	0	0	0	0	2	2
11. Gene Up-Regulation	0	0	0	0	0	0	1	1
12. Radiotherapy	0	0	0	0	0	1	1	2
13. Apoptosis Induction	0	0	0	0	0	1	0	1
<b>TOTAL GENE TRANSFER PROTOCOLS (THERAPY, MARKING and NON-THERAPEUTIC)</b>	107	7	97	5	8	348	85	*657

**\*Note:** The total number of protocols classified in the above table does not equal the total number that has been or will be reviewed by the RAC. Protocol 9903-295 has been withdrawn; Protocol 9907-331 was replaced by protocol 0004-393; Protocols 9910-347 and 9910-348 have been withdrawn; Protocol 9910-349 was replaced by protocol 0010-427; Protocol 0001-374 was replaced by protocol 0007-407; Protocol 0001-375 was replaced by protocol 0010-425; Protocol 0001-377 has been withdrawn; Protocols 0001-383 and 0001-384 have been withdrawn; Protocol 0107-492 was replaced by protocol 0110-499; Protocol 0207-547 has been withdrawn.

- Review Level 1 = Full RAC review + NIH Director approval + FDA Investigational New Drug (IND) approval. This review process is no longer in effect.**
- Review Level 2 = Accelerated RAC Review + NIH Office of Recombinant DNA Activities (ORDA) Approval + FDA IND Approval. This review process is no longer in effect.**
- Review Level 3 = Sole FDA Review Recommended by NIH/ORDA. Simultaneous submission to NIH(ORDA) required for the purpose of data monitoring and adverse event reporting. This review process is no longer in effect.**
- Review Level 4 = Sole FDA Review [submission to NIH(OBA) not required]. This is only for non-NIH funded (either direct or collaborative) institutions who elect to submit to NIH(OBA) under voluntary compliance.**
- Review Level 5 = Received by NIH(OBA). Review level pending.**
- Review Level 6 = Not Selected for RAC Public Review. Submission to NIH(OBA) required for the purpose of data monitoring and adverse event reporting. This review process is currently in effect.**
- Review Level 7 = Full RAC discussion + FDA approval. This review process is currently in effect.**